NEW OPPORTUNITIES IN TREATING KIDNEY DISEASE

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INVESTIGATIONS AND OVERSIGHT

OF THE

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NEW OPPORTUNITIES IN TREATING KIDNEY DISEASE

TUESDAY, OCTOBER 12, 1982

HOUSE OF REPRESENTATIVES,
COMMITTEE ON SCIENCE AND TECHNOLOGY,
SUBCOMMITTEE ON INVESTIGATIONS AND OVERSIGHT,
Washington, D.C.

The subcommittee met, pursuant to call, at 11:05 a.m., in the Rhodes Hall Auditorium, Ohio State University, Columbus, Ohio, Hon. Albert Gore, Jr., presiding.

Mr. GORE. The subcommittee will come to order. It is a great pleasure to be here today on the campus of Ohio State University, the great Buckeye State. Ohio State University has an excellent reputation and has repeatedly proven itself to be among the leaders in medical research, particularly in the area of kidney disease research, the focus of today's hearing.

Before we begin, I would like to say that I'm especially pleased to be able to join with my good friend and valued member of the subcommittee, Congressman Bob Shamansky, in welcoming our distinguished—we ourselves are guests of Ohio State. We would like to welcome our guests in this hearing and our witnesses.

I specifically want to thank Dr. Manuel Tzangournis, dean of the College of Medicine, and Dr. Edward H. Jennings, the president of Ohio State University, for making these facilities available, and we look forward to seeing Dr. Jennings in about an hour or so.

I would like to thank Dr. Tzangournis for the tour of the facilities as well. It's really quite a remarkable institution you have here. I say that as an alumnus of Vanderbilt University, and we feel some pride down there, but I must say these facilities are really something else. And we have had very few hearings in Washington during the past 2 years when we have not heard of some expert or experts from Columbus, Ohio, and Ohio State.

You have in Bob Shamansky a tireless advocate of this area and this institution and he has succeeded in convincing me and our colleagues on the Science and Technology Committee that this is, indeed, a hub of science and technology in the entire world and we are pleased to see it firsthand today.

And the issue that brings us here is an extremely important one. Kidney and urinary tract disease is one of the United States most serious medical and public health problems. It continues to present a difficult challenge to the biomedical research community. Each year diseases of the kidney and urinary tract cause the death of over 77,000 Americans. End-stage renal disease, as its name im-

plies, is not a single disease but, rather, it is the final common pathway resulting from a variety of different diseases. It is a point where still little hope remains for slowing the unhalting progression of disease that many unfortunately must suffer.

Prior to enactment of the medicare renal disease program, the decision to provide treatment was often influenced by financial considerations because of the enormous costs involved. In addition, because so few individuals could afford treatment and so few hospitals could afford to provide it, there were shortages in renal disease equipment, trained personnel, and treatment facilities. In short, the medical profession knew how to aid victims of renal disease, but many were going untreated because of lack of financing.

Congress responded to the expressed concern and brought to the American people as part of the amendments to the Social Security Act in 1972 the end-stage renal disease program, or ESRD. The program extended medicare coverage to every individual under the age of 65 who was medically determined to have chronic renal disease.

The program has been a success in many ways, but in terms of economics, it has shifted the financial burden from the individual to the Federal Government, and the program has continued to grow until it reached the present yearly cost of $2 billion for the medical care coverage of 76,800 individuals.

So today we face the same difficult quandry we faced before. As a society, we must again weigh a life which is beyond price against finite resources and high social costs required to provide lifesaving treatment.

The executive branch of the Federal Government, through the Health Care Financing Administration, has chosen to respond to the present situation by proposing to lower the reimbursement level for the end-stage renal disease program. This is one way to cut costs. However, the result could be the closing of many small dialysis centers. We cannot allow any renal disease patients to lose access to treatment.

I have seen absolutely no indication of any less commitment on the part of the Congress or the American people for the continued care for those with this terrible disease. While we must strive to bring the rapidly growing budget of the Federal Government under control, we must continue to support persons who depend on the Federal Government for lifesaving medical support.

Today's hearing will focus on issues that represent a different approach to the predicament we face. The subcommittee recognizes that the United States has maintained leadership in many fields of science, especially in biomedical technology. Here at the campus of Ohio State University, we turn to those who have proven to be leaders in this area for insight into new opportunities in the treatment of kidney disease. We look forward to hearing the views of our distinguished witnesses.

And now it is a great pleasure for me to recognize the ranking member of our subcommittee, Mr. Shamansky.

Mr. SHAMANSKY. I want to thank you, Mr. Chairman, the other members and the staff of the Subcommittee on Investigations and Oversight of the House Committee on Science and Technology for coming to the Ohio State University, my alma mater, especially

during the congressional recess, to investigate the skyrocketing costs of treating kidney disease.

These economic costs are forcing us to review treatment procedures in an ethical context, selecting from various sick people who is to be treated and who should not be. How is it possible to do so ethically? Are there technologically sound but cheaper alternative treatments? In effect, this matter becomes one of choosing life or death.

It is fitting that Ohio State University should host this hearing since Ohio State is a proven leader in various fields of medicine. Its work in renal disease research is well recognized in the medical community.

The end-stage renal dialysis program initiated by Congress in 1972, as you noted, provides a unique example of the Government's ability to save lives. Because the cost of dialysis is prohibitive for most people, approaching $20,000 a year or more, and because failure to obtain treatment is fatal, Congress required that dialysis be provided to all individuals who suffer from end-stage renal disease.

The renal dialysis program began with 11,000 patients. It now serves 68,000 patients. Its costs accordingly have burgeoned from $229 million in 1974 to an expected $1.8 billion in 1982. Thus, demand for more control over this program's costs has increased as part of the general desire to cut Government spending.

Yet, must we as legislators choose between saving lives and cutting Government expenditures? Or can we find new and cheaper alternatives to the dialysis program? I think it is fitting that the Investigations and Oversight Subcommittee review the achievements of the program and investigate options for its future.

When Congress initiated the kidney treatment program, it explicitly rejected the ability to pay as a test for the allocation of life-saving medical technology. As a businessman, I generally adhere to the free market philosophy, which rests on the ability to pay for what one needs and wants. Yet, in this case Congress decided that using that measure to supply such services as kidney dialysis was intolerably unjust.

Again, Mr. Chairman, I want to thank you for bringing this subcommittee to Ohio State University and I trust our findings will be beneficial to the committee, to Congress, to the taxpayers of this country, and especially to those with life-threatening kidney disease.

Mr. GORE. I have no doubt that they will. Thank you very much.

Our first witness is Dr. Manuel Tzangournis, dean and acting vice president for health services, College of Medicine, Ohio State University.

Dr. Tzangournis, we are delighted that you are able to present testimony to us. Without objection, the entire text of your statement will be included in full in the record and we invite you to proceed with your testimony.

STATEMENT OF DR. MANUEL TZANGOURNIS, DEAN AND ACTING VICE PRESIDENT FOR HEALTH SERVICES, COLLEGE OF MEDICINE, OHIO STATE UNIVERSITY

Dr. TZANGOURNIS. Thank you.

Congressman Gore, Congressman Shamansky, members of the committee, ladies and gentlemen, on behalf of our university I would like to welcome you and thank you for the opportunity to present testimony on improving treatment, research and cost efficiency in the field of kidney disease. We are pleased to offer information to the U.S. Congress on the medical advances, research efforts, ethical issues, and the costs related to kidney disease, an ailment involving thousands of persons.

When the program was enacted by Congress in 1972, you have already heard that the costs reimbursable from the Federal Government for treatment of kidney disease were projected at approximately $250 million.

Presently this figure reaches almost $2 billion. In 1981, $368 million were expended for inpatient treatment and transplantations, $653 million for outpatient and followup care, $262 million to physicians and suppliers and $4.6 million in home health and skilled nursing home facility care.

Many medical and technological advances have occurred in the past decade, permitting many Americans to lead comfortable and useful lives. In 1981, 1,458 living donor transplants and 3,400 cadaver-donated transplants were performed nationally. Dialysis patients totaled well over 50,000 nationally. And 67,000 patients were trained and moved into a home dialysis care setting.

At the Ohio State University Hospital, 60 kidney transplantations were performed in 1981. Dialysis patients also continued to increase in numbers. Annually, we treat 60 to 70 new patients in our dialysis unit and train approximately 60 patients for home dialysis.

Research and training activities are indeed an important part of our faculty efforts in this large and modern medical center. Obviously, the costs overall and efforts associated with caring for patients with end-stage renal disease either by dialysis or transplantation are enormous. The Federal Government has been principally responsible for these costs being met. But as we all know, there is no limitless source of funding.

Accordingly, it is extremely important to use resources efficiently, ethically and expertly. Most importantly, we must continue to advance our knowledge by research and teaching to prevent many of the renal diseases. For these reasons, we are appreciative of the opportunity to offer our views to you. Thank you.

Mr. GORE. Thank you very much.

Mr. SHAMANSKY. Yes. Doctor, I am curious as to what the medical profession, especially at a teaching hospital such as this, what is the profession talking about amongst itself? What are you talking to your colleagues about? What is your idea for a solution because—I say "a solution." That assumes a problem. I think there is a problem, the cost factor.

I calculated that the ratio of the number of patients treated to the cost allocated by medicare comes out to something like 40 to 1. It depends on which numbers you are using. But it is a great disproportion. Where do you see us going?

Dr. TZANGOURNIS. I think that is exactly what some of our experts will tell you. Obviously, in this particular university-based medical center, we are interested in research that will prevent

some of those renal diseases. And obviously, that is the best way to save the taxpayers and patients a great deal of money.

Mr. SHAMANSKY. I think for the record, we should note that Congressman Gore and I were given a tour of the research facilities, which is an integral part of the hospital operation, to see where potential improvement might be made in the treatment of renal disease.

Dr. TZANGOURNIS. Right. And the second role that I think this medical center plays is in training young physicians to be very proficient and recognize renal disease and prevent some of the end-stage results.

Finally, I think there are technological advances in kidney transplantation and even in dialysis, home training and so on, that I think will at least help us in our overall effort for maintaining reasonable costs.

Mr. GORE. I would just note that the figures available to the subcommittee—we will get some confirmation on this from later witnesses, I hope—but apparently, less than 1 percent of the amount that we are spending for treatment of end-stage renal disease is being spent on research into the causes of renal disease and ways to prevent renal disease.

And if we are facing the bioethical issues that are triggered by the cost crunch, it certainly makes sense for the Federal Government to do more research to try to prevent the disease and deal with its causes. And really what we are exploring here today is Congressman Shamansky's argument that there are a bunch of smart people in Columbus, Ohio, who can help the Federal Government save money by doing research and learning to better treat the disease.

So it is an honor to have you with us and we appreciate your contribution here this morning.

Dr. TZANGOURNIS. Thank you very much.

Mr. GORE. Our next two witnesses we are particularly pleased to have with us, Dr. Lester B. Salans, director of the National Institute of Arthritis, Diabetes, and Digestive and Kidney Diseases at the National Institutes of Health of the Public Health Service in the Department of Health and Human Services.

Doctor, if you would join us. And Dr. Nancy B. Cummings, associate director for kidney, urologic, and hematologic diseases. We are delighted to have you with us this morning. Thank you for traveling from Washington to this hearing. Without objection, the entire text of your testimony will be put into the record in full and, Dr. Salans, we will recognize you first and then Dr. Cummings, I understand you do not have a statement, but will be prepared to respond to questions.

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STATEMENT OF DR. LESTER B. SALANS, DIRECTOR, NATIONAL INSTITUTE OF ARTHRITIS, DIABETES, AND DIGESTIVE AND KIDNEY DISEASES, NATIONAL INSTITUTES OF HEALTH, PUBLIC HEALTH SERVICE, U.S. DEPARTMENT OF HEALTH AND HUMAN SERVICES, ACCOMPANIED BY DR. NANCY B. CUMMINGS, ASSOCIATE DIRECTOR FOR KIDNEY, UROLOGIC, AND HEMATOLOGIC DISEASES, AND CAROL FELD, OFFICE OF PROGRAM PLANNING

Dr. SALANS. Thank you very much, Mr. Chairman, Mr. Shamansky. We are delighted to be here. The last time I was in Columbus, Ohio, was in the 1950's when I came as a college student from a place called Ann Arbor, Mich., to a football game between two well known universities.

Mr. SHAMANSKY. Where is that? I don't know where that is.

Dr. SALANS. And we lost.

Mr. SHAMANSKY. I know where it is now.

Dr. SALANS. I am happy to be here with you and to have the opportunity to address the committee.

Mr. Chairman, I would like to summarize my full statement, which, if I may, I will submit for the record. I am pleased to testify on behalf of the Department of Health and Human Services. As Director of the National Institute of Arthritis, Diabetes, and Digestive and Kidney Diseases, which we refer to as NIADDK, I will focus today on an overview of kidney research for which the Institute bears lead responsibility within the Federal Government.

As you already noted, Dr. Cummings has accompanied me today. I am also pleased to introduce to you on my left, Mrs. Carol Feld, who is in our Office of Program Planning.

Before I begin, Mr. Chairman, I want to inform you that I was appointed Director of the NIADDK on June 17 of this year, so I'm relatively new to this job. But as the new Director, I am dedicated to developing and fostering creative programs for support of research on the kidney and especially on those kidney diseases that lead to end-stage renal disease.

As the subcommittee is aware and as you stated, kidney and urinary tract diseases are among this country's most serious medical and public health problems. They are common. They are associated with increased morbidity and mortality, and are an enormous burden on the economy.

The human costs of chronic renal failure or end-stage renal disease [ESRD] are a major national concern. Many different types of kidney disease lead to end-stage renal disease. Each of these diseases is characterized by a progressive downhill course which leads to irreversible kidney failure or ESRD. At one time this condition was universally fatal. However, the development of chronic maintenance dialysis has enabled us to preserve the life of affected patients for many years. And kidney transplantation provides an additional means of saving lives.

NIADDK and other NIH research has contributed substantially to the development of such maintenance therapies.

In 1981, the Federal Government spent an estimated $1.5 billion for maintenance dialysis treatment and kidney transplantation primarily through medicare. The cost to the Government of support-

ing these therapies has increased dramatically, as others before me have testified. For example, since 1974, it has been estimated that the cost has increased by more than 800 percent.

The single factor that is most responsible for this increase in cost is the progressively increasing number of new patients with end-stage renal disease entering into this program each year.

Now, while the search for improved budgeting techniques and for cost-saving innovations in the delivery of ESRD treatment is necessary and important, the most cost-effective way to reduce the cost of this program in our opinion—and the ultimate goal—is to discover ways to prevent or to arrest the diseases that lead to chronic renal failure. The number of people entering the ESRD program can be reduced. This can be accomplished through research. It is in advances produced by biomedical research that the greatest hope for individuals whose lives are profoundly affected by kidney disease rests.

As the Director of the National Institutes of Health recently testified before Congress, and I quote, "The fundamental approach to discovery at NIH continues to be through support of investigator-initiated research into life processes."

Mr. Chairman, the NIADDK is committed to this philosophy with respect to kidney disease research. This approach has been recommended and is supported by the kidney scientific community. A recent NIADDK-initiated study entitled "Research Needs in Nephrology and Urology," involving over 100 scientific experts from throughout the United States, underscored the need for more fundamental biomedical research into the physiology and pathophysiology of kidney disease, since so little is known about the underlying causes and mechanisms of the diseases that lead to ESRD.

Consistent with this recommendation, Mr. Chairman, NIADDK efforts in kidney disease research reflect increased emphasis on investigator-initiated basic research, proposed and conducted by scientists at universities and medical schools throughout the country, such as Ohio State University.

At NIH we frequently speak of a research continuum in which basic knowledge provides the foundation for applied and developmental research whose fruits are eventually translated into the practice of medicine and, therefore, into better health care.

The emergence of dialysis as an effective ESRD treatment is an excellent example of this continuum at work. Biomedical research supported by NIADDK was instrumental in the development of an understanding of how the kidney functions in the normal state and of how normal function is deranged in various diseases leading to end-stage renal disease. The Institute was then able to support research which permitted these abnormalities to be circumvented through the development and application of new therapeutic technologies such as dialysis.

Now, following the widespread application and support of dialysis treatments in the United States and other developed countries, industry is undertaking an increasingly larger share of research and development efforts to perfect dialysis techniques. This role of industry seems entirely appropriate and is consistent with this administration's efforts to enhance cooperation between the public and private sectors.

Clearly, dialysis is a significant medical achievement that has prolonged and enhanced many lives, as has renal transplantation. But it it important to realize that these are "halfway technologies" that deal only with the consequences of the disease or postpone death. Because dialysis can only partially compensate for lost kidney function caused by disease processes, it cannot restore patients to their normal lives. Dialysis is also accompanied by significant morbidity and suffering. And as you know, its cost to the economy is enormous.

Consequently, one of the highest priorities of the NIADDK is to support basic research that can unravel the underlying causes of the diseases which lead to irreversible kidney failure so that we can arrest or prevent them and ultimately eliminate the need for dialysis and the consequent costs.

This basic-research approach is vital to individuals as yet unaffected by kidney disease, who could be spared the rigorous and demanding nature of dialysis, the trauma of transplant surgery and the frequently devastating effects of immunosuppressive treatment used to prevent kidney transplant rejection. Moreover, we believe basic research is the most cost-effective way to reduce the enormous economic burden of the Federal end-stage renal disease program, since it holds promise of decreasing the large numbers of patients entering that program each year.

At the same time, we recognize the need for research aimed at enhancing treatments for the thousands of patients who already suffer from end-stage renal disease. Thus, we at NIH place a high priority on research on dialysis and transplantation so that the clinical management of existing ESRD patients can be improved.

NIH devotes about $90 million annually to a comprehensive kidney-related research effort.

In 1981, the NIADDK supported approximately $15.6 million in ESRD-related research, of which about $1.8 million was related to research on maintenance therapies.

The NIADDK's programs support basic and clinical research aimed at gaining an understanding of the normal structure and function of the kidney, and the causes, mechanisms, and natural history of the conditions that lead to kidney disease. For example, fundamental research in immunology has shed light on the immunologic mechanisms of kidney disease.

Basic research in infectious disease and hypertension has uncovered kidney infections and hypertensive renal disease as significant causes of end-stage renal disease. Antibiotic and antihypertensive drug therapy has greatly reduced these causes of end-stage renal disease. We have learned that certain analgesics and other toxic substances can damage the kidney and that their avoidance can prevent these causes of end-stage renal disease. These are but a few examples of how our programs of support of basic and clinical research on the causes and mechanisms of the diseases that lead to ESRD allow researchers to devise means to prevent and/or treat effectively these various diseases.

We recognize, however, that even if this goal were realized tomorrow, a large population of end-stage renal disease patients would still be in need of treatment advances that only research can provide. Consequently, the NIADDK supports research on mainte-

nance therapies, transplantation and the pathophysiology of uremia. Our research in these areas has led to several advances which have significantly increased the length and quality of life for the patient with ESRD.

Research and development supported by the NIADDK in the area of dialysis has led to considerable progress, including the use of single-needle dialysis, the development of specific adsorbents for uremic wastes and the introduction of portable dialyzers. As was noted earlier, further refinement of dialysis equipment is now being undertaken primarily by industry.

The NIADDK is also supporting an increasing amount of research on renal transplantation which, if successful, may provide patient rehabilitation greater than can maintenance dialysis and at a considerably lower long-term cost. The commitment of the Institute to transplantation research is reflected in intensified efforts in this area for which NIADDK funding has increased from $1.9 million in 1979 to $3.5 million in 1981. This expanded research in transplantation has contributed to several promising developments, including an enhanced understanding of the immunologic mechanisms involved in the rejection of transplanted kidneys and the demonstration that Cyclosporin A, a new immunosuppressive agent, can substantially increase kidney graft survival, compared to previous regimens.

At the same time, NIADDK research on the complications of renal disease has led to several significant advances in the management of the uremic syndrome of ESRD. For example, vitamin D has been shown to be an effective therapy in bone disease of ESRD patients.

Mr. Chairman, these are only a few examples of research approaches and advances in the area of kidney disease. Through this comprehensive approach of basic, clinical and applied research and development on the kidney, the treatment of existing end-stage renal disease can be improved while the search is intensified for new scientific knowledge upon which prevention is based. Compelling research opportunities now exist for gaining new insights into the underlying causes of kidney disease, immunologic aspects of kidney dysfunction and the role of nutritional, metabolic, and other factors in the progression of kidney disease. We will explore these and other research opportunities in our quest for new scientific knowledge, the most potent weapon against kidney disease.

Two new initiatives currently in the planning phase will serve to illustrate the continued commitment of NIADDK to research on end-stage renal disease.

The first initiative involves studies on the natural history and mechanisms of kidney diseases leading to ESRD. The planned studies would evaluate different groups of patients, focusing on the role of nutritional, metabolic and other variables in the disease process. Investigations could include the effects of low protein diets, low phosphate diets and lowered glucose levels on the workload of the kidney.

The second initiative focuses on quantitative indices for initiating dialysis. Widespread consensus does not yet exist concerning when dialysis should be initiated for various patient populations. The NIADDK initiative would encourage investigators to look at a

range of objective criteria, including nutritional, metabolic, and other factors. The studies planned could thus provide important new knowledge to enable physicians to determine more precisely in any given patient when dialysis should be initiated and to tailor dialysis better to individual patient needs.

The NIADDK is enthusiastic about both of these initiatives and believes that the basic and clinical research that they stimulate will contribute important new knowledge.

Mr. Chairman, this overview touches only briefly on the impact of kidney diseases, the vigor of NIH efforts in this area and advances and opportunities that flow from the Nation's investment in biomedical research. Our highest priority in this area is to support investigator-initiated basic and clinical research into the etiology and pathophysiology of various kidney diseases, particularly those leading to irreversible kidney failure.

Through research supported by NIADDK and NIH, effective methods have been developed to reduce deaths in the current ESRD population with dialysis and transplantation therapies. Now, the major effort in kidney research must be directed at understanding, arresting and ultimately preventing kidney disease, so that we can reduce the human and economic toll borne by affected individuals and by our entire society.

Mr. Chairman, my colleague and I would be pleased to answer any questions you or other members of the subcommittee may have.

Thank you.

[The prepared statement of Dr. Salans follows:]

PREPARED STATEMENT BY LESTER B. SALANS, M.D.

Mr. Chairman and Members of the Subcommittee:

I am pleased to testify on behalf of the Department of Health and Human Services at this hearing on new opportunities in the treatment of kidney disease. As Director of the National Institute of Arthritis, Diabetes, and Digestive and Kidney Diseases (NIADDK)--a component of the National Institutes of Health--I will focus today on an overview of kidney research, for which the Institute bears lead responsibility within the Federal Government. Accompanying me today is Dr. Nancy B. Cummings, the Institute's Associate Director for Kidney, Urologic and Hematologic Diseases.

In providing an overview of the Federal role in kidney research, I will briefly review the magnitude of the health problem presented by kidney disease, the relationship of research to kidney treatment, and the strong commitment to kidney research at the National Institutes of Health. Finally, I will highlight several research advances and opportunities in kidney research.

Before I begin, Mr. Chairman, I want to inform you that I was appointed Director of the NIADDK on June 17, 1982. As the new Director of this Institute, I am committed to providing the kind of creative and vigorous leadership required of this most important area of national health research. As part of this approach, I am dedicated to developing and fostering programs for support of research on the kidney.

Magnitude of the Health Problem

As the Subcommittee is aware, kidney and urinary tract disease is one of this country's most serious medical and public health problems. It is

common. It is associated with increased morbidity and mortality, and it is an enormous burden on the economy.

Based on our analyses of kidney and urinary tract mortality data of the National Center for Health Statistics, it is estimated that each year, diseases of the kidney and urinary tract cause the death of over 77,000 Americans. On a nationwide basis, the direct medical cost of kidney disease is estimated at $5.5 billion annually. There is also an estimated $2.6 billion annual cost to the economy as a result of lost productivity due to death and disability.

Within the broad area of kidney disease, the human and economic costs of chronic renal failure or end stage renal disease (ESRD) are a major national concern. Many different types of kidney disease lead to ESRD. These kidney diseases are characterized by a progressive downhill course which leads to the final common pathway of irreversible kidney failure. At one time, this condition was universally fatal. However, the development of chronic maintenance dialysis has enabled us to preserve the life of affected patients for many years. More recently, kidney transplantation provides an additional means of saving lives. The development of such technologies was contributed to by NIADDK and other NIH research. Grantees of this Institute pioneered maintenance dialysis therapy by contributing through research to the development, refinement and use of the artificial kidney machine. At the same time, we and others supported fundamental and applied research which led to our current ability to utilize kidney transplantation.

Over 60,000 Americans were maintained by dialysis treatments in 1981, while over 21,000 new patients with irreversible kidney entered dialysis

programs. During 1981, over 4,800 patients received kidney transplants.

In 1981, the Federal Government spent an estimated $1.5 billion for maintenance dialysis treatment and kidney transplantation, primarily through Medicare. The cost to the Government of supporting these therapies has increased dramatically, since Congress authorized funding for the End Stage Renal Disease Program through Public Law 92-603.

The single factor that is most responsible for the increase in costs of the federally supported ESRD program is the progressively increasing number of new patients with ESRD entering into this program.

While the search for improved budgeting techniques and for cost-saving innovations in the delivery of ESRD treatment is necessary and important, the ultimate goal is to prevent or to arrest the diseases that lead to chronic renal failure. This is a goal to which the NIADDK and the NIH are firmly committed. Biomedical research holds the greatest hope for the human beings behind the statistics--the individuals whose lives are profoundly affected by disabling and often life-threatening kidney diseases.

Clearly, concerted efforts are needed among relevant Government agencies, the scientific community, voluntary health organizations, and industry to combat all forms of kidney disease.

Relationship of Research to the Treatment of Kidney Disease

As the Director of the National Institutes of Health recently testified before Congress, "The fundamental approach to discovery at NIH continues to be through support of investigator-initiated research into life processes." This approach is particularly relevant to kidney research since so little is known about the underlying causes and mechanisms of

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kidney diseases such as glomerulonephritis, polycystic kidney disease, and diabetic nephropathy, the main precursors of ESRD.

I would like to emphasize that this is the Institute's philosophy with respect to kidney disease research. Such an approach has been recommended and is supported by the kidney scientific community. In the late 1970's, NIADDK initiated a broad study, "Research Needs in Nephrology and Urology," involving over 100 scientific experts from throughout the U.S. A primary goal of this study was to identify impediments to the understanding of fatal kidney diseases. This study underscored the need for more fundamental biomedical research into the physiology and pathophysiology of kidney disease. Consistent with this recommendation, NIADDK kidney efforts reflect increased emphasis on investigator-initiated basic research, proposed and conducted by scientists at universities and medical schools throughout the country, such as Ohio State University. Over 67% of the NIADDK budget is directed toward investigator-initiated research grants, approved by the scientific peer-review system. Thus the scientific community itself identifies promising research ideas for the Institute to pursue.

At NIH, we frequently speak of a research "continuum," in which basic knowledge provides the foundation for applied and developmental research, whose fruits are eventually translated into the practice of medicine and the delivery of better health care. This is a dynamic process, in which new knowledge gained at each step benefits all components of the health research and health care system.

The emergence of dialysis as an effective ESRD treatment is an excellent example of this "continuum" at work. Biomedical research supported by

NIADDK was instrumental in the development of an understanding of how the kidney functions in the normal state, and how normal function is deranged in various diseases leading to ESRD. The Institute was then able to support research which permitted these abnormalities to be circumvented through the development and application of new therapeutic technologies, such as dialysis.

Now, following the widespread application and support of dialysis treatments in the United States and other developed countries, industry is undertaking an increasingly larger share of research and development efforts to perfect dialysis techniques. This role of industry seems entirely appropriate, and is consistent with the Administration's efforts to enhance cooperation between the public and private sectors. While industry focuses primarily on enhanced commercial application of dialysis, NIADDK continues to support research to improve these technologies, with particular attention to identifying ways to avoid or reduce complications experienced by dialysis patients.

Clearly, dialysis is a significant medical achievement that has prolonged and enhanced many lives, as has renal transplantation. But, it is important to realize that these are "halfway technologies," that deal only with the consequences of the disease or postpone death. Because dialysis can only partially compensate for lost kidney function caused by disease processes about which we have limited understanding, it is palliative and cannot restore patients to their normal lives. It is also accompanied by significant morbidity and suffering, and its cost to the economy is enormous.

Consequently, one of the highest priorities of the Institute is to support basic research that can unravel the underlying causes of the diseases which lead to irreversible kidney failure--so that we can arrest or prevent them, and ultimately eliminate the need for dialysis.

This basic-research approach is vital to individuals as yet unaffected by kidney disease, who could be spared the rigorous and demanding nature of dialysis, the trauma of transplant surgery, and the frequently devastating effects of immunosuppressive treatment to prevent kidney transplant rejection. Moreover, we believe basic research is the most cost-effective way to reduce the enormous economic burden of the Federal End Stage Renal Disease Program, since it holds promise of decreasing the large numbers of patients entering that program each year.

In order to be able to arrest or prevent diseases leading to chronic renal failure, we must expand the fundamental knowledge base about normal kidney function and about the mechanisms of kidney disease responsible for the relentless downhill course of kidney function that can occur after an initial insult has produced tissue damage. NIADDK is dedicated to the support of basic research that will increase this knowledge base. Such knowledge is essential to developing innovative and effective therapies to halt progressive loss of kidney function and, ultimately, to preventing the onset of kidney disease.

At the same time, we recognize the need for research aimed at enhancing treatments for the thousands of current ESRD patients. Thus, we give a high priority to research on dialysis and transplantation, so that the clinical management of existing ESRD patients can be materially improved.

I would now like to review, in greater detail, NIH and NIADDK efforts in kidney research.

NIH Commitment to Kidney Research

The National Institutes of Health's mission, broadly stated, is to uncover new knowledge that will lead to better health for everyone. In the kidney area, NIH devotes about $90 million annually in a comprehensive kidney-related research effort.

Since time does not permit me to describe fully these kidney research activities, I would like to focus primarily on ESRD research supported by NIADDK. In 1981, the Institute supported approximately $15.6 million in ESRD-related research, of which about $1.8 million related to research on maintenance therapies.

Arrest or prevention of kidney diseases leading to ESRD is the Institute's ultimate goal. The Institute supports basic and clinical research aimed at gaining an understanding of the normal structure and function of the kidney and the causes, mechanisms and natural history of the conditions that lead to kidney disease, so that means can be devisd to prevent, arrest or treat effectively the various diseases of the kidney that lead to ESRD.

We realize, however, that even if this goal were realized tomorrow, a large population of ESRD patients would still be in need of treatment advances that only research can provide. Consequently, the Institute supports research through an active Chronic Renal Disease Program, with subdivisions focusing on maintenance therapies, transplantation, and the pathophysiology of uremia. In this program, research is under way on

the technologic aspects of the different dialysis modalities, on organ preservation and immunosuppressive therapy in kidney transplantation, and on the complications of chronic renal disease or uremia.

Uremia frequently affects many systems of the body including the central nervous system, peripheral nerves, the cardiovascular system, the gastrointestinal tract, the state of nutrition, and bone and blood tissues. Thus the Institute supports a great deal of research on the causes and mechanisms of these uremic complications and on developing therapeutic approaches for preventing or reducing their severity. Our research in in this area has led to several advances which have significantly inproved the quality of life for the patient with ESRD.

The Institute is also supporting an increasing amount of research on renal transplantation, which, if successful, may provide patient rehabilitation greater than maintenance dialysis can, and at considerably lower long-term cost. The commitment of NIADDK to transplantation research is reflected in intensified efforts in this area, for which NIADDK funding has increased from $1.9 million in 1979 to $3.5 million in 1981. This expanded research in transplantation has contributed to several promising developments including an enhanced understanding of the immunologic mechanisms involved in the rejection of transplanted kidneys, and the demonstration that Cyclosporin A, a new immunosuppressive agent, can increase substantially kidney graft survival, compared to previous regimens, and is associated with decreased infections in experimental animals and in patients. A randomized, controlled clinical trial, using Cyclosporin A, already has shown evidence of: 1) improved graft function, 2) fewer episodes of graft rejection, 3) fewer infectious complications,

and 4) decreased hospitalization, with consequent decreasing costs. Another advantage of Cyclosporin A is that it appears effective in low doses and thus may not be as toxic as other immunosuppressive drugs. If these early clinical results are shown correct in the long term, there is great promise for improved graft survival in kidney transplantation.

The Institute's commitment to research on ESRD and to seeking new and creative approaches to generate new knowledge is perhaps well illustrated by two new initiatives we are planning.

The first initiative involves studies on the natural history and mechanisms of kidney diseases leading to ESRD. Following an initial insult to the kidney, some patients experience rapid loss of renal function, other experience slow deterioration, and others remain relatively stable, with little or no progression of disease. The planned studies would evaluate these different groups of patients, focusing on the role of nutritional, metabolic and other variables in the disease process. Within the area of nutrition/metabolism, investigations could include the effects of high/low protein diets; high/low phosphate diets; and high/low glucose levels on the workload of the kidney.

The second initiative focuses on quantitative indices for initiating dialysis. Widespread consensus does not yet exist concerning when dialysis should be initiated for various patient populations. The NIADDK initiative would encourage investigators to look at a range of objective criteria, including nutritional, metabolic, and biochemical variables, as well as degree of anemia and central nervous system involvement. The studies planned by NIADDK could thus provide important new knowledge to enable

physicians to determine more precisely, in any given patient, when dialysis should be initiated, and to tailor dialysis better to individual patient needs.

The Institute is enthusiastic about both of these initiatives and believes that the basic and clinical research that they stimulate will contribute important new knowledge.

Now, I would like to turn for a moment to just one example of NIADDK research on non-fatal kidney and urinary tract diseases, about which few data are available. NIADDK-supported epidemiologists recently published a study of a defined population in northern California, in which, of the major kidney and urinary tract diseases, the highest incidence rate was that of benign prostatic hyperplasia, with urinary tract infections next highest, and urinary tract calculus (stones) third.

Benign prostatic hyperplasia (BPH), can be responsible for damage to the kidneys themselves if the prostate grows enough in size to obstruct urinary outflow. Because the incidence of this disorder increases with age so that it can affect about 80% of men by age eighty--and because 10 percent of men afflicted will require surgery--research efforts to find possible medical therapies are under way. Advances have been made in hormonal and biochemical studies of specific prostate tissues, and two distinct types of tissues, stromal and epithelial, have been separated. Hormone receptors have been identified and inter-relationships of sex hormones and their effects upon prostate tissue are being delineated.

Research Advances and Opportunities

I would now like to highlight several advances and opportunities illustrating the contributions that biomedical research can make to improve health and well-being. Advances include:

- o Two-decades of progress in antibiotic therapy that has aided in the treatment of kidney infections and thus reduced a significant cause of ESRD.
- o Development of anti-hypertensive drugs that can prevent progressive and ultimately fatal hypertensive kidney disease, one of the major conditions that in the past has led to ESRD.
- o Demonstration that a Vitamin D metabolite (1,25-dihydroxy Vitamin D_3) is an effective therapy in the bone disease of ESRD patients.
- o Demonstration that long-continued use of certain analgesics can lead to progressive interstitial nephritis, which is ultimately followed by ESRD.
- o Significant progress in the understanding of immunologic mechanisms of renal injury, including the recognition that immune complex deposits can form locally in glomeruli and evidence that cell-mediated mechanisms can lead to glomerular injury.
- o Demonstration that three-year kidney graft survival is significantly greater in transplant patients who have been given one or more blood transfusions. One-year graft survival in recipients given 10 pre-transplant transfusions has been shown to be almost twice that of patients receiving none. This beneficial effect, assessed in a series of over 500 patients, has been noted to increase with the number of pretransplant transfusions and is greater for more mismatched grafts.
- o Findings supporting the hypothesis that sustained hyperfiltration (or some hemodynamic determinant(s) of hyperfiltration) is ultimately detrimental to glomerular structure and functions.
- o Development of a streamlined technique for evaluation of stone-forming patients, which simplifies the outpatient work-up and reduces cost over 75%. Development of medical treatment of kidney stones for some stone types (uric acid and calcium).
- o Development of a new immunosuppressive drug, Cyclosporin A, which appears to be promising in improving the survival of transplanted kidneys and other organs as compared with conventional post-transplant immunosuppressive therapy.

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- o Observation that hemodialysis patients who undertook exercise training showed improvement of abnormal lipid and glucose metabolism, lowering of elevated blood pressure levels, and improvement in their anemia. It is theorized that the markedly decreased levels of physical activity in uremic patients may be responsible for some of their observed metabolic abnormalities.

Let me also cite several technologic developments relevant to kidney disease. Of course, continued development of dialysis methods is the technologic area that bears most directly on the treatment of ESRD. Research in this area has led to considerable progress including the use of single-needle dialysis, the development of specific absorbents for uremic wastes, and the introduction of portable dialyzers. As noted earlier, further refinement of dialysis equipment is now being pursued primarily by industry. At the same time, NIADDK research on the complications of renal disease, dialysis techniques, and transplantation therapies is an important corollary to the Institute's fundamental studies on the kidney. Through this comprehensive approach, the treatment of existing end stage renal disease can be improved while the search for new scientific knowledge is intensified.

In addition to dialysis, many other technologies have been developed that are helping to increase our fundamental knowledge by facilitating a wide number of investigations. Such was the case when micropuncture was developed so that fluid could be obtained from the renal tubule. The ability to isolate segments of the renal tubule and study effects of the surrounding solutions upon its function has further increased knowledge about renal cellular behavior during the production of urine.

Micropuncture techniques are used to measure the pressure of the microscopic blood vessels leading to the glomerulus, the kidney's microfilter, and to study its filtrate. Using these techniques, scientists have studied animals with experimental diabetes or whose kidney tissue has been surgically removed. These studies on the effect of abnormal states on the progression of kidney disease implicate elevated pressure in microscopic vessels in the disease process.

Another technologic development involves application of nuclear magnetic resonance (NMR) to studies of the kidney and of the salt-secreting gland of the dogfish. These studies are providing new information about cellular transport processes applicable to the kidney and to other organs. Because of the special significance of salts in kidney and heart disease, the ability to study their movements is of great importance.

Technologic developments are also playing an important role in research on urinary tract stones. Because of the high incidence of such stones, mentioned earlier, and the relative paucity of research into this disorder, NIADDK initiated Specialized Centers of Research (SCORs) in Urolithiasis in 1977. In the five years since inception of this program, we have witnessed marked increase in research activity, a burst of knowledge about some underlying disease mechanisms, and the development of new treatment approaches.

For example, a streamlined technique for initial evaluation of stone-forming patients was developed, which made a simplified outpatient work-up possible at about one-sixth the cost of the more cumbersome inpatient

work-up. Analytical methods were also developed for microanalysis of oxalate in biological fluids, a component present in 75% of kidney stones. Medical treatment of patients with elevated urinary levels of uric acid and of calcium has been devised, using readily available medications (allopurinol and hydrochlorthiazide). This therapy, over a long-term follow-up period, has been shown to decrease the number of stones formed in chronic patients followed for over three to four years. An innovative technique developed in Germany for destruction of stones, in vivo, using high energy shock waves has stimulated interest in developing of comparable techniques at lesser cost in the United States. At the same time, basic research continues into metabolic abnormalities underlying stone formation.

These are but a few of the scientific and technologic advances that are paving the way for the pursuit of additional research opportunities and for the development of more effective therapeutic approaches to kidney disease. Compelling research opportunities now exist with respect to gaining new insight about the underlying causes of kidney disease, immunologic aspects of kidney dysfunction, and the role of nutritional, metabolic, and other factors in the progression of kidney disease. NIADDK will explore these and other research opportunities in our quest for new scientific knowledge, the most potent weapon against kidney disease.

Conclusion

Mr. Chairman, this overview touches only briefly on the prevalence and impact of kidney disease, the vigor of NIH efforts in this area, and

advances and opportunities that flow from the nation's investment in biomedical research. Our highest priority in this area is to support investigator-initiated basic and clinical research into the etiology and pathophysiology of various kidney diseases, particularly those leading to irreversible kidney failure. Through research supported by NIADDK and NIH, effective methods have been developed to reduce deaths in the current ESRD patient population with dialysis and transplantation therapies. Now, the major efforts in kidney research must be directed at understanding, arresting and ultimately preventing kidney diseases, so that we can reduce the human and economic toll borne by affected individuals and by our entire society.

Mr. Chairman, my colleague and I would be pleased to answer any questions you or other members of the subcommittee may have.

Mr. GORE. Thank you very much.

Let me ask a couple of questions.

Can we come up with a better name for your institute that will resolve itself into an acronym of some kind?

Dr. SALANS. We had one until the Institute's name was recently changed. At one time we were called NIAMDD, but that recently was changed and now we are called NIADDK. I understand that there is a move to change it again and I am not sure whether that will make it easier or harder.

Mr. GORE. Something like Buckeye or something?

Dr. SALANS. That would be appropriate when you are visiting Ohio.

Mr. GORE. Let me ask you this: You state in your testimony that each year these diseases will cause the deaths of over 77,000 persons, is that correct?

Dr. SALANS. Well, I think the correct figure is about 45,000 or 50,000 deaths annually from ESRD. I think there are about 70,000 people who are in the end-stage renal disease program now, is that correct?

Dr. CUMMINGS. There are 77,000 total deaths from all types of kidney and urinary tract disease, which includes cancers.

Mr. GORE. I see. All right.

Dr. CUMMINGS. That is a 1975 figure. We don't have a current one because it takes quite a while to get the final data.

Mr. GORE. Well, I would imagine that some of those cancers resolve themselves into ESRD?

Dr. SALANS. That's correct.

Mr. GORE. Do they not?

Dr. SALANS. Yes.

Mr. GORE. Well, in any event, the direct medical costs of kidney disease will approach $5.5 billion with an added indirect cost of

$2.6 billion. I guess the basic question is: Isn't there more that could be done to prevent this enormous tragedy?

Dr. SALANS. Well, I think, Mr. Chairman, that there certainly is a considerable amount more that can be done in research on kidney disease, as well as many other diseases that affect our population. We believe that adequate funding is essential in order to undertake these efforts. Recognizing the reality of the current economic constraints and diminishing resources, I think it is important that we in the Department of Health and Human Services and at NIH set our priorities wisely and manage our programs with maximum efficiency and effectiveness. And we believe we have done so and are doing so.

We believe the President's budget supports the best research and the best researchers in kidney diseases possible at this time.

Mr. GORE. Well, I appreciate that response but, you know, the recognition of present financial constraints cannot preclude the recognition of future financial constraints as well. And with the rapid growth in the cost of the program, it would seem to a layman like me that maybe it would make sense to spend more than one percent of HCFA's expenditures on ESRD on ways to prevent it and improve treatment. I suspect that you may partly agree and partly be constrained in agreeing too enthusiastically, but let me just put the question to you.

Dr. SALANS. Well, all I can say, Mr. Chairman, is that I think that under the present circumstances we are making our best effort to accomplish the objectives that you have laid out. We think that we are doing a pretty good job on that.

I have just described to you two new initiatives that the NIADDK plans to undertake under the President's 1983 budget to address very specifically the problem that you raise. We believe that those two initiatives, which mean a commitment of additional funds to support those areas of research, will enable us to understand better the causes of kidney diseases and how those diseases progress to end-stage renal disease and will enable us, through research, to reduce the cost to both the country as a whole and to the individuals who suffer from kidney diseases.

Mr. GORE. Well, I certainly hope you are right.

Let me ask you about the transplantation option. Last year, during 1981, more than 4,800 kidney transplants were conducted in the United States. Your testimony indicates that the kidney transplant is the most cost-effective method of treatment of renal failure for those patients for whom it's appropriate.

Can you tell us why that number is so small relative to the number of persons undergoing dialysis?

Dr. SALANS. Mr Chairman, it is correct that the number of kidney transplantations in this country has leveled off over the last several years. My understanding is that there has been an increase of approximately 12 percent in the number of transplantations since 1979. That certainly is well below what had been expected originally.

As I understand it, one of the major reasons that transplantation has not increased more has been because so far the major type of kidney transplantion, that is, transplantation with cadaver kidneys, has not been as successful as we would like. This form of

transplantation comprises about 75 percent of all renal transplantation and is associated with a 1-year survival rate of approximately 50 to 60 percent, depending on the center preforming the transplant surgery. Those in whom cadaver transplantation is not successful then have to go back on dialysis.

So with less than ideal success of cadaver kidney transplants physicians may be reluctant to refer patients for such procedures.

Second, I think that the number and quality of suitable organs available for transplantation have been and continue to be fairly limited. A number of factors account for this, but it is clear that we need to improve our procurement procedures and that we need to have a better-educated public, particularly community hospitals, about the importance of the procurement system and the importance of kidney transplantation to the public health. I think these are the major reasons why tranplantation has not increased.

Now, let me briefly say, on the other hand, that I am optimistic. Number one, I have indicated to you that there have been significant advances in transplantation research, one of the most striking being the discovery and development of a new immunosuppressant, Cyclosporan A, which seems to have an enormous advantage over what we have had before to suppress the immunorejection of the transplanted kidneys. That has, I think, the potential of changing the practice of kidney transplantation as well as transplantation of other organs.

In addition, we are learning a lot more about tissue typing, the importance of tissue typing for successful transplantation and the need to match more closely the tissue types, the genetic types of the transplanted patient and the donor. And we have shown that with with attention to this we can improve the success of cadaver transplantation. So I think that there is hope for the future and, if we can improve the procurement system, we will see an increase in the number of transplants in this country.

I hasten to add, however, that from my viewpoint that does not get at the heart of the problem, which is understanding the causes of the diseases that lead to the need for transplantation and dialysis.

Mr. GORE. So one of the things that you would say that we need to encourage is more kidney donors and more kidneys being made available and, at the same time, that we encourage and support the research to make the procedure safer and better?

Dr. SALANS. I believe that is the case.

Mr. GORE. Let me ask you about uremia. Could you tell me just very briefly, either you or Dr. Cummings, what we are talking about there?

Dr. SALANS. Dr. Cummings, why don't you address that question.

Dr. CUMMINGS. Uremia refers to a symptom complex and the name refers to the fact that there is a high level of urea in the blood.

But this symptom complex, in the context of end-stage renal disease, is a multisystem set of symptoms secondary to the fact that the kidneys are not functioning normally. The kidney has several functions. The main one that causes difficulty is that of excretion and maintaining the interior milieu of the blood, and then there are also hormonal functions that are served by the kidney.

Mr. GORE. Now, this is improved by dialysis, correct?

Dr. CUMMINGS. Parts of it are. Dialysis is an empirical therapy and it improves some aspects of uremia, the ones that are actually life threatening. Patients treated by dialysis over a long period of time, some up to 17–20 years, will develop other complications, such as bone disease and anemia, which are persistent, Cardiovascular disease is accelerated.

Mr. GORE. Well, I am told—and I want you to correct me if I am wrong—that we do not know—that science does not know what it is that dialysis removes from the blood that improves the uremia, is that correct?

Dr. CUMMINGS. That is partially correct and partially incorrect. The life threatening aspects of end-stage renal disease are ability to control certain compounds in the blood, such as salts containing potassium.

If potassium is elevated to a certain critical level, it can then make the heart stop. In that sense, we know what dialysis does in removing potassium and we know how it balances salts.

But there are a number of metabolic compounds that are removed, and perhaps some that are taken out that should not be taken out because they are necessary, which dialysis acts upon. We do not know what those are.

There are probably 2 or 3,000 compounds that are present in uremic patients, some necessary and some deleterious, that are removed by dialysis.

Dr. SALANS. May I add that normal kidney makes a number of very critical substances. One, for example, is something that stimulates the formation of blood cells in bone marrow. Dialysis does not make up for the things that are missing.

Dr. CUMMINGS. Also, treat vitamin D metabolite to which Dr. Salans referred and another hormone that have an effect on bone disease in uremia also receive final treatment in the kidney. When there is no functional kidney tissue left, there is no substitute for that.

Mr. GORE. I just realized I pronounced your name wrong. Dr. Salans, is that correct?

Dr. SALANS. Yes.

Mr. GORE. I am sorry that I mispronounced it earlier.

If you have 2 or 3,000 compounds in the blood of an uremic patient, that is an awesome challenge to scientific research, is it not?

Dr. CUMMINGS. There once was a time when people were looking for "a uremic toxin" in patients with end-stage renal disease. They decided that that sort of "fishing expedition" is not very valuable, that it is better to try to understand the normal functioning, and then some of the abnormal functioning, through looking at biochemical and physiologic pathways. In that way, perhaps one may find those pathways that are deranged that may be responsible for the symptomology.

Mr. GORE. Well, the reason they gave us on the other approach had to do with allocation of scarce research resources, correct?

Dr. CUMMINGS. No. It turned out that it was not a very profitable avenue to take and that there seemed to be better ones: to look at specific symptoms that are deranged and to try to find out what is

going on there and then perhaps see what affects these derangements.

Mr. GORE. I see. Do you want to go now? Mr. Shamansky.

Mr. SHAMANSKY. Thank you, Mr. Chairman.

Doctor, in my original remarks, I gave you a wrong number. It is still not a good number, and I will tell you what I mean.

Medicare payments for ESRD patients are expected to reach approximately $2 billion for fiscal year 1983. That is the fiscal year we are in now. This would be for a coverage of 76,800 persons.

In 1979, ESRD expenditures took 4.13 percent of the total medicare expenditures for 0.19 percent of the persons treated through medicare funds.

Those are the last figures I have available to me. That is 21 to 1. What we are worried about is the disproportion between the expenditures for the number of people treated. I also have figures that project that the 1983 costs will be a little over $2 billion, as we mentioned.

In 1987, it will be a little over $3 billion. That is an increase of, say, 50 percent in the cost. But the number of people anticipated to be treated would increase by only 17 percent, so there is the disproportion between the 17 percent in the number treated, but an increase in costs of 50 percent.

Now, they are even allowing for an increase in inflation.

Can you tell me, assuming validity of all in these numbers, why the disproportion?

And second, to address immediately, you talked about an 800 percent increase over the years in the costs. But if the number of patients that has been treated has been increased 800 percent, then on a per-unit cost, it may not be increasing. I would like you to address the economics of that.

Dr. SALANS. Well, the numbers I think that we are all working with are relatively soft. Actually, the folks who deal with the numbers—HCFA—may be able to give you firmer data than I can.

But it is my understanding, that the cost of dialysis per patient has not been increasing over the years. It has been relatively stable.

The increased cost of the program is, as we have indicated, the direct result of an increase in the number of people that are entering into the program.

The explanation for the disparity or discrepancy that you are describing—and we do not know really exactly what the nature or the magnitude of that disparity is—probably lies in the fact that, as Dr. Cummings just stated, there are associated with dialysis a number of complications.

It may well be that the cost of the complications associated with dialysis and, therefore, their management, the morbidity that is associated with the treatment itself, may be enhancing the costs above what anybody had anticipated before.

Patients are living longer. Dialysis is being applied to patients who are aging. I think all of those factors may explain some of the magnitude of the disparity.

Mr. SHAMANSKY. You mentioned in your testimony the increase in funds available for research in transplantation, correct?

Dr. SALANS. Yes.

Mr. SHAMANSKY. But you did not mention whether or not you had a proportionate increase in other aspects of your work. Overall, what is your budget doing?

Dr. SALANS. We have increased our support of research in renal transplantation, as I indicated to you, from about $1.9 to about $3.5 million since 1979. If you would give me a minute, I can give you some idea, in our institute, what has happened.

Mr. SHAMANSKY. With renal now?

Dr. SALANS. Pardon?

Mr. SHAMANSKY. I am talking about renal now, not the other diseases.

Dr. SALANS. Yes, for kidney disease during roughly that same period. In 1979, Mr. Shamansky, our institute allocated approximately $33 million in support of research on kidney disease.

In 1981, we supported approximately $42 million in kidney disease research in our institute alone.

As I have indicated, other NIH institutes also support kidney research, so that the total amount of NIH support for this kind of research is higher than just NIADDK support. Dr. Cummings has just stated that in 1976, research at NIH on the kidney was supported at a level of approximately $56 million. As I said in my testimony, it currently stands at about $90 million. So it has gone up substantially.

Mr. SHAMANSKY. I cannot forget that we are taking this testimony at a research hospital and I would like to ask if NIH has a policy and a procedure to help steer research in directions that they think are most relevant.

When you go to allocate these studies, what criteria are you using these days?

Dr. SALANS. Well, NIH determines its priorities for research in a variety of ways. We, of course, respond to the concerns and issues of the public as expressed through the Congress. Our policies are determined by the Congress and the executive branch.

We, also respond to public concern as expressed through other mechanisms, such as our advisory boards and lay and professional organizations with whom we interact at all times.

We rely heavily on investigators in the field to identify in their applications for research grants the scientific opportunities, the gaps and the needs in various areas. We try to determine with our colleagues in the scientific community whether there is a critical mass of information, a basic knowledge with which we can then launch a research effort.

Mr. SHAMANSKY. Doctor, would that be a standard operating procedure for an institute such as the NIH?

Dr. SALANS. That is correct.

Mr. SHAMANSKY. My question is more particularly, are you changing that or is it just that what you have been doing you continue to do?

Dr. SALANS. Well, it changes constantly because the next step of that process is, once having identified that there is a need or an opportunity, we then go through a variety of mechanisms to solicit from the scientific community applications for research in areas for which there is a need or an opportunity.

The scientific community then submits applications that are reviewed through our scientific peer review mechanisms, which select out those investigators and those meritorious ideas which are most likely to be responsive to the public needs as we have described.

Those applications that come into the system are changing constantly because the state of the art is changing constantly. The state of technology is changing constantly.

Mr. SHAMANSKY. If I may pursue that, because this is something that has at least concerned me certainly since I have been on the committee, granting what you have just said—and that sounds very logical to me—it still comes down to the fact that when you make an award for an investigation to an institution, such as Ohio State, it really consists of two parts, correct, direct costs and indirect costs?

Dr. SALANS. That is correct.

Mr. SHAMANSKY. And the problem is—as long as you are here, we may as well discuss these questions—why the disparity in indirect costs where it seems to me you get a situation that a high indirect cost institution, which I believe I am safe to say Ohio State, I believe, is one of those—in other words, what you are getting for your dollar?

How do you allow one institution with a very high indirect cost rate to get an award as distinguished from one with a low indirect cost rate, because the net amount applied to direct research is obviously diminished to the extent indirect costs are appreciably higher?

Dr. SALANS. Well, there certainly are discrepancies; there is quite a variability in the amount of indirect costs that NIH allows from one institution to another. And a variety of factors apparently contribute.

Mr. SHAMANSKY. Apparently——

Dr. SALANS. They have to do with variations in certain regional costs, for example.

Mr. SHAMANSKY. Well, OK, but I am concerned about this "apparently" business. I am concerned with what steps you are taking as an institution, a Federal institution to, say, correct or really justify that kind of a disparity because this really raises some questions.

Dr. SALANS. Well, you are absolutely right. And as you are well aware and so is Congressman Gore, NIH, and the administration have been very concerned with this issue.

Mr. SHAMANSKY. I know you are concerned but what are you doing about it? That is what I want to know.

Dr. SALANS. We are in the process now of studying the whole indirect cost procedure and how indirect costs are arrived at, what are the factors that are responsible for the high degree of variability in direct costs that institutions recover from NIH and how best to make the program more efficient and much more effective.

As you are well aware, it was suggested that in 1983, at least while a study of the whole indirect cost question was going on within the Department of Health and Human Services, indirect costs be reduced by a certain amount as a holding pattern until we could develop a better and more efficient way of doing it. That does

not appear to be happening and it probably will not happen in 1983.

But we are continuing a concerted effort within the Department of Health and Human Services and within NIH to try to develop a more effective and more efficient way of looking at this issue.

Mr. SHAMANSKY. We, as Members of the Congress, are concerned with cost effectiveness and I hate to see a situation in which the apparently least efficient are benefited the most.

Dr. SALANS. That is something that the Department of health and Human Services and NIH is very concerned about and this is certainly what we are after in the study that we are undertaking.

Mr. SHAMANSKY. To pick up from that, I have what is represented as a couple pages from, I think, one of President Reagan's favorite publications, the Readers Digest. And this is from the November 1982 issue. On page 149, "Kidney Dialysis, A Taxpayer's Nightmare."

And they have been reviewing generally over here if the cost per patient, often over $25,000 annually—you are getting down to something like $500 a week—if you go in 3½ hours a day for 3—over 3—on 3 different days—I hope my numbers are right—approximately $45 an hour, why is there such a disparity in the costs of these clinics that frankly have sprung up?

You know, I know there is a need for a clinic, but I am concerned about the disparity in the cost—and obviously, some of them are rip-offs. There is just no question about that. What is the Institute doing about that?

Dr. SALANS. Mr. Shamansky, I think that the question you asked is certainly one that is on everybody's mind. I just would remind you again that that is an area for which NIH, through its support of research, does not have primary responsibility.

Mr. SHAMANSKY. What you are saying is that is somebody else's responsibility?

Dr. SALANS. It is the responsibility of all of us, I think, as public citizens, to be concerned about that.

But in terms of the explanation for the differences that you point out, one needs to look to those Federal agencies with discrete responsibility in this area, not to the NIH.

Mr. SHAMANSKY. I am raising the question frankly. This is why I welcome the opportunity to have this exchange with you. It seems to me that it would be logical for you to set criteria, some guidelines. You must have some insight.

Certainly, I would be willing, if I were a State regulator, State legislator, to—we need some objective source for saying what reasonable costs should be.

And for instance, you know, the numbers, the magnitude of difference from one operator to another leads you clearly—and as this article points out, they load in some of the most outrageous costs, jet planes and all those nonsensical things that end up raising the costs which it takes money away from the treatment of the patients.

It seems to me it would be appropriate for the NIH, while you say it is not direct research, I think you at your peril close your eyes and say, "Oh, that is somebody else's responsibility."

We are here to help you. That is what this hearing is about. But it seems to me there is a legitimate role there for the NIH to address that issue.

It seems to me—I made some notes earlier. I do not see in the study I have done in preparation for this hearing that there really is—at least I am not aware—if there is, my apologies to everyone—there does not seem to be a review, a peer review for the appropriateness for dialysis in a particular case.

And you alluded—you did comment on that there are no standards as to when you initiate—and some of my reading indicated that there is just one standard treatment and you said, "Well, we are going to look into the business of tailoring the dialysis treatment for the patient," which seems to me to be very logical but it raises the question: Why was it not raised before?

Is there any idea of having a peer review? You have tissue committees in hospitals. Don't we have treatment committees in dialysis centers, both proprietary and public in the sense of Ohio State?

Dr. SALANS. Mr. Shamansky, there are a number of questions that you have asked.

Let me try to address as many as I can as briefly as I can.

First of all, the responsibility for the areas that you have addressed primarily—that is, cost control, setting standards for reimbursement, et cetra—really does fall within the Health Care Financing Administration and not the NIH. Recently, Mr. Fountain held hearings with regard to that whole issue.

Mr. SHAMANSKY. But from the scientific point of view, I am asking you, can you help them set those standards, accepting the fact that——

Dr. SALANS. Standards of cost?

Mr. SHAMANSKY. No.

Dr. SALANS. I think we would have great difficulty setting cost standards.

Mr. SHAMANSKY. I am not talking about that. I am simply saying standards criteria that would indicate when treatment is appropriate and inappropriate.

Dr. SALANS. I think that one of our initiatives, one of the new initiatives that we have just described to you is very responsive to the suggestion that you are making. Our intent is to get experts in the field of kidney disease who do research on and treat patients with kidney disease to come together and try to establish a set of guidelines for when dialysis should be initiated. We can then go to HCFA or they can ask us——

Mr. SHAMANSKY. Sure.

Dr. SALANS [continuing]. What recommendations does the scientific community, does NIH through the scientific community, or the scientific community through NIH have for us in that regard?

We can provide them with that information. We think that is a very valuable undertaking.

Based on that, HCFA then can hopefully develop regulatory guidelines for reimbursement which will address the very specific issues that you describe.

Mr. SHAMANSKY. Do you personally have an opinion on the idea of peer review?

Dr. SALANS. We use peer review constantly, continuously and we think it works very well for our research effort. PSRO and other kinds of mechanisms may be useful peer review mechanisms for the practice of medicine.

Mr. SHAMANSKY. Do you think it appropriate for the initiation or the maintenance of dialysis?

Dr. SALANS. I am not sure what the administration's viewpoint on that is.

Mr. SHAMANSKY. I didn't ask you that. I asked you your own personal opinion.

Dr. SALANS. I think that the quality of delivery of health care certainly should very well be under some kind of peer review. I think it usually is.

In this specific area, I think one needs to look very carefully—and I think that is being done within the Department of Health and Human Services and probably at the level of Congress as well—at how best to accomplish that within the particular area.

Mr. SHAMANSKY. I have just one last question.

In Washington State—and again I quote from the Reader's Digest, which is an impeccable source in this case, you understand—in Washington State, where nephrologists encourage home care, 54 percent of these patients are on home dialysis. Yet, nationwide, only 17 percent of patients dialyze themselves.

Can you comment, assuming that is true, what is your experience and your knowledge with respect to home dialysis and its future?

Dr. SALANS. Well, I think that home dialysis is a very important and effective part of our armamentarium in the management and the maintenance of these patients. It certainly is less expensive than dialysis.

Mr. SHAMANSKY. Where are we going with it?

Dr. SALANS. As we are gaining more experience, its use is being expanded. Home dialysis is not a treatment for everyone. While there are benefits, there are also certain disadvantages to this method of therapy that certain people do not like.

Having home dialysis means that you must have somebody home with you and managing you constantly, and that in itself creates certain hardships.

But I think that in terms of cost effectiveness, home dialysis in that sense may have real advantages for some.

Mr. SHAMANSKY. Is there a precedent of a combination of home dialysis, instead of three trips to the renal dialysis center, they would come in 1 day a week here and 2 days of the week at home?

Dr. CUMMINGS. There are limited-care facilities, and the Veterans' Administration has been a leader developing these. And what they provide is a center to which the patients come and basically do almost all the work themselves, but there is someone there to help them.

Mr. SHAMANSKY. Just—and since President Jennings has just arrived—is there any research done on an artificial kidney to be put in the body?

Dr. SALANS. Not to my knowledge.

Mr. SHAMANSKY. OK. I was just curious.

Dr. SALANS. I think it is important in regard to your questions, sir, to point out that all methods of management, all modalities really have to be tailored to the individual needs of the patient. Home dialysis may well be much more desirable for some than for others.

Mr. SHAMANSKY. Thank you very much, Dr. Salans.

Mr. GORE. Let me just say briefly, first of all, I think what Congressman Shamansky has been heartfully pointing out is that there are a number of reasons—that when we confront this cost crunch—to look to institutions like Ohio State where you have the ability to make more subtle judgments about scarce resources and how to deal with the disease.

Let me just explore one more of those briefly. Currently dialysis treatment provides a uniform type of dialysis regardless of the age of the patient, the weight or size of the patient, is that correct?

Dr. SALANS. Why don't I ask Dr. Cummings to respond to that.

Dr. CUMMINGS. That isn't true. Some centers have a rough, standard way of dialyzing, but most of the dialysis is somewhat tailored to the patient's needs, and it varies from time to time. There may be some centers that have a standard prescription, but in general, physicians still look at their patients as individuals and know whether they need longer dialysis or special kinds of dialysis.

Mr. GORE. But in other words, in an institution like this one, you would be more likely to find the tailored treatment program but there may be more of a standard treatment for all patients regardless of size or age in some of the less sophisticated centers?

Dr. CUMMINGS. I don't think I could comment on that.

Mr. GORE. Yes. OK.

What about the Institute's plans for nutrition research and the relationship between diet and the progression of renal disease?

Dr. SALANS. Well, we have plans for that. The NIADDK, as well as NIH in general, recognizes the critical role of nutrition in health and disease, including kidney disease.

It has been an integral part of the medical management of patients with end-stage renal disease for a long time, and it has made the management prior to the availability of dialysis and transplantation possible.

We know, for example, that it is important to regulate protein intake. We also know that it is important to regulate potassium and sodium intake, et cetera.

It has been hypothesized that if one paid more attention to the nutritional management of the patient with end-stage renal disease, there could be several potential advantages.

One, the rate of progression of the disease might be slowed, the underlying disease from an early course to one that progresses on to end-stage renal disease.

Another advantage is that the need for frequent dialysis might be reduced.

Nutritional therapy might also have the effect of delaying the need for dialysis.

However, there really isn't any convincing evidence through controlled clinical studies to support these hypotheses. In fact, there are a number of concerns that experts in the kidney field throughout the world have raised with regard to the use of nutrition to

prevent or delay the need for dialysis or decrease the frequency of dialysis.

Nevertheless, there is no question that nutrition is an important part of the management of kidney disease, and it may play a very important role in the natural history, the progression of the disease to the terminal phase.

Therefore, the NIADDK pays very close attention to nutrition. We are doing a number of things in that area. We are currently supporting a significant amount of research in nutrition in end-stage renal disease.

The NIADDK is currently supporting about 38 projects related to nutrition in kidney disease in the amount of about $5.5 million. We intend to do more.

Second, we have just had within the last 6 months or so a workshop attended by a variety of experts from throughout the world specifically to address the question of the relationship between nutrition and kidney disease.

They made a series of specific recommendations about what research NIH ought to be doing in this area. The Institute agrees with those recommendations and has incorporated them into both of the new initiatives that I described to you in my opening testimony. So, we take this area of nutrition and ESRD very seriously. We intend to follow up on this. And we are in the process now of planning very carefully for how to go about this.

Dr. Cummings, in the next few months, is convening a group of experts in both nutrition and kidney disease to help us plan this first phase of solicitation.

Mr. GORE. Well, that is excellent. And as you may know, I have had a special interest in this area and got an amendment attached to the authorization bill this past year and money in the continuing resolution to target this area, and I look forward to working with you in the future.

I want to thank you again for really excellent testimony and for your appearances here today. It has really contributed a great deal to our understanding of this problem. Thank you very much for meeting with us.

Dr. SALANS. We thank you for the opportunity to meet you.

Mr. GORE. I would like to welcome the president of Ohio State, Edward Jennings, and if we could go off the record for a minute, I haven't met President Jennings, and I would like to.

[Discussion off the record.]

Mr. GORE. We will go back on the record now, and I would like to welcome you, President Jennings. And let me thank you for your excellent hospitality and tour of the facilities we have had here, and I would like to call on Congressman Shamansky to further introduce you.

Mr. SHAMANSKY. Well, I am happy to say that President Jennings represents a new and important era at Ohio State. I am old enough to say it is good to have a younger man aboard because there are great challenges and it is going to take a different experience and a different outlook, and so far I can say with assurance that you have met the challenge.

We know that we are not going to have as many Federal funds available and I think that we are going to look more toward our

own resources within the State and the various business and other communities available to us. And I know that will be part, a great part of your challenge. We have talked about that.

And, of course, part of my job is to offer certainly my assistance to you in that course of action. We would be grateful to you if you would have something to say, I am sure, on what we are doing here.

STATEMENT OF EDWARD JENNINGS, PRESIDENT, OHIO STATE UNIVERSITY

Mr. JENNINGS. Well, thank you Bob, and Congressman Gore.

First of all, I would like to say how flattered we at Ohio State are that you would hold your hearings on our campus. We often recognize nationally the greatness of this institution but sometimes in the community we are not a profit in our hometown.

Coming from the academic community, I know of the excellence of Ohio State in many areas of the institution. In the medical area, of course, you are on a very critical issue here in kidney dialysis, but I think it goes well beyond that because it reflects not so much a money issue, but a need in all parts of the country and the world to recognize the vital importance of basic research in our society in general.

All of us can stop and think of examples of basic research that was seemingly irrelevant, unproductive, even silly at the time that has led to some of the great breakthroughs in medicine, in engineering, physics, and chemistry all over the world.

And we need only cite some examples of Goddard and the space activity and Jonas Salk in the vaccine, even our own people here developing a feline leukemia vaccine that represents a major breakthrough of developing immunity without dealing with the life or attenuated virus associated with that.

All of this and probably my great fear well beyond the money is the tendency we all have to today forget the enormous importance of basic research associated not only with our institution but with the Federal Government and with the private sector in this country.

And we need to be certain that in our zeal to measure absolute results, that we always keep in mind the long-term but enormously productive benefits of basic research.

I know you share that and I know you do, Bob, as well. And I think your coming here reflects your understanding of the importance of basic research in an institution. And I appreciate that very much. And I know my colleagues from the faculty do. I see my colleagues from the university hospitals.

You might not realize, but we are in the process right now of the initial planning for a 160-bed research facility directed at cancer. And Ohio State should in the future become one of the Nation's premier cancer research institutions with the addition of this $40 million structure that is going—that is in the planning stages right now and will be built immediately east of Rhodes Hall.

I want to thank you for coming and I know you want to hear from people who know what they are talking about in this——

Mr. SHAMANSKY. We have been doing that just now.

Mr. JENNINGS [continuing]. And I don't.

Mr. GORE. Thank you very much. And you weren't here at the beginning of the hearing, so I want to repeat it for you. In the last 2 years, every science and technology issue that has come before our subcommittee, we have heard about the role played by Ohio State, and I might add the others, Battelle Institute, other institutions here in Columbus, Ohio.

And Bob has succeeded in convincing me of the central role played by Ohio State. And if I wasn't convinced before, I certainly am after seeing your facilities today and having a chance to talk with your researchers here. And we appreciate your hospitality.

Mr. JENNINGS. Well, thank you very much. And we know you support us and we are very pleased that you are here and we look forward to working more with not only you but NIH. And we understand occasionally that research grants have to go to some other smaller, not quite as good institutions like Stanford in Chicago——

Mr. SHAMANSKY. I would never agree with that.

Mr. JENNINGS [continuting]. But when they come to the Ohio State University, we will all be happy. Thanks a lot.

Mr. GORE. Thank you very much.

We have a panel of witnesses now, and I would like to invite them to come to the witness table.

Dr. James Cerilli, professor and chairman of the department of surgery at Albany Medical College in Albany, N.Y.; Dr. Mark Mentser, director of dialysis at Children's Hospital in Columbus; Dr. Lee Hebert, professor of medicine and director of the division of renal diseases here at Ohio State, accompanied by Dr. Ronald Ferguson; and finally, Dr. Samuel Gorovitz, professor of philosophy in the department of philosophy at the University of Maryland.

Gentlemen, I would like to thank you all for coming here. Without objection, the prepared statements of all our witnesses will be put into the record in full at this point, and if you all care to summarize any portions of your statements, feel free to do so.

We would like to begin with Dr. Cerilli, professor and chairman of the department of surgery at Albany Medical College in New York. Doctor, welcome, and please proceed.

STATEMENTS OF JAMES CERILLI, M.D., PROFESSOR AND CHAIRMAN OF THE DEPARTMENT OF SURGERY, ALBANY MEDICAL COLLEGE, ALBANY, N.Y.; MARK MENTSER, M.D., DIRECTOR OF DIALYSIS, CHILDREN'S HOSPITAL, COLUMBUS, OHIO; LEE HEBERT, M.D., PROFESSOR OF MEDICINE AND DIRECTOR OF THE DIVISION OF RENAL DISEASES, OHIO STATE UNIVERSITY, ACCOMPANIED BY DR. RONALD FERGUSON; AND SAMUEL GOROVITZ, PROFESSOR OF PHILOSOPHY, DEPARTMENT OF PHILOSOPHY, UNIVERSITY OF MARYLAND

Dr. CERILLI. Well, thank you very much, Congressman Gore, Congressman Shamansky. It is appropriate, I think, that I might mention that my relationship to this institution was for over 15 years working in the transplantation program here and I appreciate that you appreciate the enormous contributions that Ohio State has made to renal disease.

This is a fantastic place and doing nothing but getting better. And with the support of individuals like yourself, it is going to continue to have a major impact in the development of new ideas and the management of patients with end-stage renal disease.

Mr. GORE. Now, wait a minute. You are not on the faculty at Ohio State?

Dr. CERILLI. No. I left—as of 3 months ago, I left to assume the chairmanship of the department of surgery at Albany Medical College.

Mr. GORE. So you are an objective, disinterested witness?

Dr. CERILLI. That is correct.

Mr. SHAMANSKY. And accurate.

Mr. GORE. All right. Go ahead.

Dr. CERILLI. It is a pleasure, really, to have this opportunity to talk to you. And what I have to say, really, is an amalgamation of several statements that I have made before congressional committees, primarily the Subcommittees on Health of the House Ways and Means Committee and the Senate Finance Committee, and I would like to say that I have been extremely impressed by your questions and the background work that you have obviously done on end-stage renal disease to seek out data to help you do your job.

I had initially intended to give a little bit different talk than I am now going to give, but because of the direction of the questions that you have put forth, I think I can help you answer some of those.

My goal and my purpose, really, is to help tell you about transplantation, where it is, but particularly what problems there currently exist that I think that you can help in, and particularly address those questions that I have heard mentioned several times this morning relating to the costs of this program because they are a concern to everyone, particularly as costs undoubtedly as they have in many other countries, will ultimately lead to an influence on how we treat our patients. So that is where I intend to concentrate.

Transplantation as a discipline has evolved dramatically in the last few years. The results have improved dramatically. Utilizing certain techniques, for instance, graft survival, that is, kidney survival can now approach over 90 percent when you use kidneys from living related donors and certain immunologic manipulations.

With the advent of Cyclosporin, it looks as if the graft survival rates with cadavers, that is kidneys from nonrelated, recently deceased individuals will approximate 80 and possibly even greater percentages, an enormous change over just a few years ago.

Mortality, that is the chance of dying after transplant, has dramatically decreased. A few years ago, it wasn't unusual to have transplantation centers with 15-, 20-, and even 30-percent mortality rates. And now an acceptable mortality rate for a good transplant center should be less than 5 percent.

It is a new science, but it is a science, a clinical science that has improved dramatically in the last few years. And I don't think one can make a rational judgment as to how it should fit into the overall program of end-stage renal disease management unless you understand the current state of the science of transplantation. And

that is where we are. It is a big difference from as recently as 5 years ago.

Now, transplantation also has, in addition to the effect on mortality and morbidity, a significant impact on rehabilitation rates of patients with end-stage renal disease. There have been many studies in the literature that show a successfully transplanted patient has about three times the chance of working, supporting his family and paying taxes and is contributing to his own health care costs as does a patient on end-stage renal disease dialysis therapy.

The two therapies must work together. But nevertheless, if we say that one of our goals is rehabilitation, which I hope it is, therefore, I think we have to say that the successfully transplanted patient far and away meets that goal more than does the patient on dialysis.

Now, a third comparison is the problem of costs. Everyone is afraid and with some logic that the cost of this program will lead to a curtailment of support for patients with end-stage renal disease. And I can appreciate the very difficult job you have to allocate a very limited number of resources and the frightening statistics of the escalation of costs of this program. And you obviously, as you should, are looking for ways to curtail these costs in the management of patients with end-stage renal disease.

But I think in order to do this properly—and I am talking about the care of patients, not the prevention of the disease—I think you need to have a perspective of what those costs are. And I have done a little analysis that I have presented before that I think summarizes my view, and I think a reasonably accurate view of the relative cost effectiveness of dialysis and transplantation.

There have been several studies, including those figures presented by the end-stage renal disease program itself, the Health Care Financing Administration that have shown that the average cost of a transplant is approximately $20,000 to $25,000 with a continuing cost of somewhere around $1,000 per year, sometimes more, sometimes less, depending on the particular patient. But that is about what it is.

Now, the cost of center dialysis is about $28,000 a year, again, reasonably documented data.

Now, if you take 100 patients and you transplant them and you followed them for 10 years and you compare their costs with 100 patients that are on center dialysis and you make 2 assumptions, one, that graft survivals are about 60 percent, which is very reasonable in today's environment, and, two, that all of the costs of the failures of the transplants you ascribe not to dialysis but to transplantation, you come up with a savings of over $15 million for only 100 people over 10 years. Now, that is documentable data and I think it puts in perspective why I feel, as do many others, the cost relationships and the cost benefits of transplantation.

Now, one might then ask with this sort of background in terms of mortality, morbidity and costs, what is the best way by which these services can be delivered. And Congressman Shamansky, I think, was trying to get at this with his questions of the previous witness who testified, Dr. Salans.

I think there is good data published, amongst other things, in the New England Journal about 2 years ago by a Dr. Luft from Stan-

ford that showed that the mortality rates and the chance of success when delivering complicated health services is better in large, sophisticated medical units than it is in the smaller units. He presented, I think, overwhelming data to show that results are better and, therefore, costs are lower when you deliver sophisticated medical care in big centers.

Now, there was a study out of Birmingham, England, a few years ago that showed the results are better for transplantation in the larger units. So I think as a principle, one could support the position that if you want to be cost effective as well as medically effective, you support the concept that you deliver these services in large sophisticated medical complexes rather than fragment them willy-nilly amongst thousands of small hospitals.

Now, there is an addition to this problem of delivery. The problem of being unable to provide the services because we don't have enough kidneys. It is really discouraging to realize how fragile the whole concept of cadaver transplantation really is. It rests upon the goodwill of the public to donate a kidney because they feel they want to help someone else. And it does so with the anxieties that the individual's who dies care will not be compromised by participating in such a program. That is indeed a very, very fragile relationship and one that I fear could be very easily disturbed.

It also relies on the continuing energies of the physicians involved in transplantation and their hard-working paramedical personnel to get these kidneys. We need innovative thoughts. We need a new approach. We need the stimulus to come more and more from the public than, rather, from the medical community to get these kidneys.

About a year and a half ago, we met with Representative Crane and had him introduce a bill to try to at least not so much get this bill passed or that bill passed, but to get people thinking about new ways of getting the public to donate kidneys. And this suggestion was that you give a tax credit to the families of those patients who donated their kidneys after death.

The idea fell upon nonfertile ground for a variety of reasons. One, some people were afraid that it might be abused. But my position is that I don't think the risk of it being abused is any greater than the abuse of cadaveric procurement at all.

In the early 1960's, everybody was afraid that cadaveric procurement, removal of kidneys from recently deceased individuals, would inhabit the objective relationship between the physician and the recently deceased individual. That has not happened. It has been well policed and very carefully done. And I don't believe that with this concept it will happen either.

But we need to provide this type of innovative thinking. And this particular idea, for instance, is one in which everyone benefits. The patient benefits. He gets a kidney. The family benefits to help them financially during a very difficult time. And the government benefits because it gets patients off of very expensive dialysis.

But whether this is a solution or one like it, I don't pretend to know. But I think you need this type of thinking if we are going to get off the current foundation upon which cadaveric organ supply is based.

Congressman Shamansky addressed a very important principle, and that is who is really going to be the individual, individuals, agencies or groups that is going to be the advocate for cost effectiveness. Is it going to be the public? Is it going to be the doctor? Is it going to be the government? And if the government, at what level?

The problem right now is, is that the tendency is to remove everyone from being the motivator for cost effectiveness. The tendency through the deregulation proposed recently by the administration would actually tend to, in my mind, remove any effective overview or overseeing of cost effectiveness for the end-stage renal disease program. It aims to transfer that responsibility to the States and take it away from the Health Care Financing Administration at the Federal level.

In my view, this is a mistake. It is a mistake because the Federal Government is paying the bill. The States are not. The States have no real fiscal reason to promote in most instances cost effectiveness for this program. But the government does. It is a federally funded program. It is not a State-funded program.

And when you transfer the responsibility to the States, the decisions are based not on cost effectiveness but on many other motives. And if we are going to control the costs, we must put the responsibility for the administration together with the program where the dollars are coming from.

I realize I have touched on several different subjects, and I have tried to be reasonably specific. I hope to provoke a discussion on the issue and to help you help the patients with end-stage renal disease so we can do this job better and more cost effectively.

I appreciate the opportunity and I again would like to thank you and the other Members of Congress throughout the years who have been so supportive of this particular medical problem.

Thank you.

[The prepared statement of Dr. Cerilli follows:]

CURRENT ROLE OF TRANSPLANTATION IN THE MANAGEMENT OF END STATE RENAL DISEASE PATIENTS

by

G. James Cerilli, M.D.

During the past several years there has been marked improvement in the application of transplantation techniques for the management of patients with End Stage Renal Disease. These improvements have been widespread throughout most transplant centers but unfortunately have not been totally uniform in that there are still programs throughout the country whose current results do not meet the capability of the science. In a recent study by the American Society of Transplant Surgeons in which eight large university centers were surveyed for the period 1978-1979, an up-to-date analysis of what can be achieved with renal transplantation in competent centers was documented. Cadaveric graft survivals are now 55-60% at one year and living related donor graft survivals in that series was 75% taking all patients as a group. However, since that study, there has been an introduction of a new technique for living related donors, namely, the use of donor specific transfusions which has increased the graft survival rate in those patients receiving living related donor kidneys to over 90%. This is achieved with a sensitization rate of approximately 40% and these sensitized patients are subsequently placed on cadaveric waiting lists. Thus, the current state of the art of graft survival in 1982 should be 90% for living related donors and over 55% for cadaveric grafts. The new immunosuppresive drug, cyclosporin, holds promise for a cadaveric graft survival of over 80%.

There has been a dramatic decrease in mortality rates following transplantation in most, but unfortunately not all, centers throughout the country. The mortality rate in the American Society of Transplant Surgeons survey based on over a thousand kidney transplants was less than 10% during the first year and an insignificant mortality rate after that. These results have been improved upon in several centers such as Ohio State University where our cumulative mortality rate during the last four years has been 1½% for living related donors and 5% for patients receiving cadaveric donors. Thus, the risk from transplantation to the life of the patient has dramatically decreased. These mortality results

should be compared to that offered by hemodialysis since this is the alternative with which the patient is obviously faced. An analysis of the Northwest Kidney Center dialysis results over a several year period indicated an average mortality of approximately 6-10% per year. At Ohio State University, patients on chronic hemodialysis during the past six years had an average mortality rate for non-diabetic patients under the age of 55 and who had been on dialysis for a minimum of three months of approximately 8% per year. Thus, by the end of four years, approximately 36% of the patients who were under the age of 55 and who were non-diabetic had died. Thus, it is clear that the risk of transplantation to the patient's life is actually less than the risk of that same patient staying on dialysis.

The major goal of the treatment of patients with end stage renal disease is obviously rehabilitation. In this area transplantation is far superior. Several studies including those from the University of Minnesota, The Ohio State University, and Peter Bent Brigham, have all indicated that approximately 75-80% of patients undergoing successful transplantation are rehabilitated in terms of resuming an occupational activity equal to or exceeding that prior to their chronic illness. This is in contrast to patients on chronic hemodialysis as recently reported by the Duke University and reported in the New England Journal of Medicine where less than 1/3 of the patients are able to maintain themselves independent of support. Thus, the major goal of rehabilitation is clearly much more successfully met by utilization of transplantation techniques.

The cost of the program of management of patients with end stage renal disease is a concern for all -- the physician as well as the government who is meeting the cost. An appropriate cost analysis is, therefore, indicated to make proper decisions as to which direction funding emphasis should be placed. In addition, cost-analysis is necessary to insure that financial disincentives are not placed within the system that tend to lead to referral of patients into medically- as well as cost-inefficient forms of therapy. The current cost of a renal transplant is approximately $20-25,000 during the first year and approximately $1,000 per year afterwards. The cost of chronic center hemodialysis is approximately $150 per treatment plus the cost of hospitalizations during each year; it is estimated that the cost of center hemodialysis is

approximately $28,000 per year. These figures are the result of an analysis of data from the University of California - San Francisco, The Ohio State University, and the end stage renal disease program itself. If one transplants 100 patients and follows these patients for 10 years and assumes that 60% of the grafts succeed, the cost of this is approximately $15 million. This is ascribing all graft failures and the subsequent dialysis costs to the cost of transplantation. If one places the same 100 patients on center hemodialysis for 10 years the cost of these same 100 patients is over $28 million. Thus, there is a savings to the end stage renal disease program of approximately $13 million for only 100 patients when these 100 patients are transplanted and followed for 10 years. This does not take into consideration the additional cost-benefits from the fact that many more patients who were transplanted are rehabilitated and thus are working and are not being supported by social programs and are paying taxes to the government to help defray their medical costs. It is clear, therefore, that successful transplantation is markedly more cost-effective than is hemodialysis. The costs of home dialysis are only approximately 30% less than that of center dialysis and the current cost of chronic ambulatory peritoneal dialysis is almost equal to that of home hemodialysis. Therefore, the cost advantages of transplantation are still maintained when one compares transplantation to either home dialysis or to chronic ambulatory peritoneal dialysis.

One might ask the question as to the method by which one can optimally deliver transplantation services so as to maximize cost-effectiveness and optimize results. There is data to strongly support the position that transplantation can best be provided via a limited number of large centers that can maintain a group of experienced individuals exclusively devoted to providing transplantation services. This conclusion is based on the following data:

> A study reported in the New England Journal of Medicine by Luft of Stanford University showed that the results including the mortality rate for the delivery of sophisticated surgical services were poorer in small surgical units as compared to large units. In addition, a study from Manchester, England, demonstrated that the mortality rates and graft survivals were better in large transplantation units as compared to small transplantation units. Also, in a survey of over 81

> transplantation centers by the American Society of Transplant Surgeons, the mortality rate in small centers (those performing less than 20 transplants per year) were 40-100% greater than in centers performing large numbers of transplants (i.e., over 40 transplants per year). Thus, it is not only logical but there is substantial data to support the position that the results in transplantation are better in large units than in small units. If the clinical results are better then costs are also lower as poor results are expensive.

There remain many organizational as well as clinical problems in the field of transplantation. First of all, there is certainly an inadequate supply of cadaveric kidneys. The current system depends upon the energies and continuing efforts of transplant coordinators, physicians, and nurses to stimulate potential donors and their families to give kidneys. This system is inefficient and works intermittently. Appropriate legislation to stimulate cadaveric kidney donation is indicated. The more kidneys that are available, thus removing patients from costly hemodialysis, not only benefits the patients in terms of their medical problem but saves the federal government millions of dollars. The possibilities for such federal legislation would include the following concepts:

(a) provide an estate benefit (as via the Crane bill) for the deceased individual allowing donation of his organs;
(b) allow income tax deductions for the families; and,
(c) increase the medical coverage currently provided to the donor so that the costs of the donor's terminal illness will be covered under Medicare.

Such ideas, while appearing to increase the cost of the Medicare program for end stage renal disease would, in fact, decrease it by removing patients from hemodialysis and allowing a greater percentage of end stage renal disease patients to return to work.

A second major problem is the current trend toward the proliferation of dialysis and transplantation units -- a trend that will be accelerated if de-regulation is permitted. The proliferation of dialysis and transplantation

units will lead to a fragmentation of services making it difficult to provide optimal care because units will be so small that the proper clinical expertise cannot be concentrated. In addition, small dialysis units clearly tend to sequestrate patients and not refer them to more cost-effective forms of therapy such as home dialysis or transplantation. The smaller units, particularly those not closely associated with University centers sequester their patients because the referral of patients to transplantation or to home dialysis can be devastating to their financial solvency. Both the nephrologists as well as the center are highly dependent on the income generated by a very small group of patients and it is only by maintaining these patients on center dialysis that the hemodialysis center can continue to function in a fiscally solvent fashion. Sequestration of patients on hemodialysis is readily documented by the data submitted to Congress by Health Care Financing Administration indicating there were literally thousands of patients in the transplantable age group that are not on transplant waiting lists. While some of these show legitimate medical reasons that contraindicate transplantation, many do not. Therefore, deregulation by promoting the proliferation of units will clearly lead to the explosion of costs of the end stage renal disease program. The states will not monitor this as they have no financial stake in the cost of the program. It is easy to document multiple instances where unneeded facilities are approved by the states purely on a political basis rather than on a medical or cost-effective basis. It must be understood by those responsible for the administration of this program that to deregulate the end stage renal disease program at the federal level and to remove the federal controls currently under Health Care Financing Administration will lead to deterioration of the quality of care and an escalation of the costs under the current funding mechanism. If the system is to be deregulated, it must be accompanied by a radical change in the funding system such as a limitation in the total amount of dollars provided to each patient for the management of his end stage renal disease problems. Then, the patient will have a stimulus to seek a cost-effective form of care. If the current system is deregulated, and the funding mechanism is kept the same, there will be no individual or agency that will have as a primary goal the management of this program in a cost-effective fashion.

The reimbursement system has a large impact on referral patterns of end stage renal disease patients and the new reimbursement proposal for dialysis

will, without intent, adversely effect transplantation. The changes currently proposed in the reimbursement system for hemodialysis that bases the hemodialysis reimbursement rate on a combination of home dialysis and center dialysis patient profile will have an inhibitory effect on the referral of patients to transplantation because it will be in the financial interest of centers to refer patients to home dialysis but it will not be in the financial interest of the centers to refer patients to transplantation. It is certainly not necessary to build into the reimbursement system financial incentives to refer patients to transplantation but it is important not to build-in financial disincentives for the referral of patients to transplantation such as will occur if the proposed reimbursement system is implemented.

In summary, it is clear that transplantation has improved markedly in the past few years as a clinical discipline. Mortality rates have dropped precipitously in the better centers; graft survivals have improved dramatically for living related donors; and, have stabilized for cadaveric donors. It is also now clear that the services can be best delivered in a limited number of highly skilled centers performing large numbers of transplants to optimize the clinical result and minimize the cost. Current proposals for basing the reimbursement rates for dialysis on a combination of center and home dialysis will act as a financial deterent to the referral of patients into transplantation. The proposal to deregulate the end stage renal disease program by elimination of the impact of the Health Care Financing Administration and the Networks will be catastrophic to the delivery of these services in a cost-effective and medically-effective fashion. It is recommended that the federal controls be actually increased in order to prevent the proliferation of units which lead to the sequestration of patients on costly center dialysis. It is clear that neither proprietary dialysis services, hospitals, nor the physicians in any single dialysis unit, except those within major centers, are uniformly able to avoid the sequestration of patients because of the financial incentives to maintain patients on in-center dialysis. The removal of federal controls without the institution of a funding mechanism that will provide a mechanism for cost-effectiveness will lead to an escalation of the costs of this program and ultimately leading to a denial of end stage renal disease care for some patients.

Mr. GORE. Well, thank you for really excellent testimony. We will both have questions of you but we will wait until the other members of the panel have testified.

Our second witness is Dr. Mark Mentser, director of dialysis at Children's Hospital here in Columbus. Dr. Menster, welcome.

Dr. MENSTER. Mr. Chairman, Mr. Shamansky, as a board-certified pediatric nephrologist specializing in the care of children, I welcome this opportunity to present to the Subcommittee on Investigations and Oversight my views concerning current and future treatment of children with kidney disease.

Recognizing that children and adults share many of the same disease processes and therapeutic modalities, I will direct my comments toward the critical differences that separate the child from the "patient" with kidney disease. Children are different in their metabolic response to disease, have unique emotional needs and require a more individualized method of care.

The annual incidence of end-stage renal disease in the pediatric age group is 1 to 3 new patients per 1,000,000 population per year; accounting for approximately 5 percent of the medicare ESRD population. Congenital renal disorders, obstructive uropathy and renal dysplasia, are the major causes of end-stage renal disease in children; whereas, acquired glomerulopathies, hypertension and diabetes are the primary diagnoses in adults. Consequently, children must endure the disease process and its inherent complications during the time of rapid body growth, intellectual development and emotional maturation.

Children who are later to develop end-stage renal disease are generally diagnosed before their 10th birthday, with most being identified before 5 years of age. Growth failure is a common presenting sign of renal disease in children even at a point in time when renal function is only moderately reduced. Appropriate therapies directed toward the complications of renal failure are rapidly introduced, however, in spite of early intervention, progressive renal failure ensues.

Comprehensive care of these children is best carried out by a renal team consisting of physicians, nurses, nutritionists, and social workers. Medical therapy consists of early identification and correction of any obstructive anatomic abnormality, eradication of infection, and aggressive medical prevention of bone disease and hypertension. Nutritional guidance is provided to assure maximal caloric intake since inadequate energy utilization has been identified as one of the many factors related to growth failure.

Early psychosocial evaluation provides the renal team with an assessment of the intellectual, emotional, and financial needs of the family and better prepares all concerned for future crisis intervention. Testing the child's motor and intellectual skills permits the early identification of deficiencies and institution of appropriate therapy. Eventual development of end-stage renal disease dictates the institution of more specific therapeutic modalities, that is, dialysis and transplantation.

The goal of pediatric ESRD treatment is restoration of the normal growth and development process so that the child may develop to a healthy productive adult. Dialysis therapy, hemodialysis or peritoneal dialysis, controls but does not ameliorate the physio-

logic abnormalities; and only through transplantation is normal renal function restored and the ultimate goal achieved.

Most children undergo dialysis therapy prior to renal transplantation. Technological advances and highly skilled personnel enable small children and even neonates to undergo successful hemodialysis therapy. Treatment-related complications including precipitous drops in blood pressure and convulsions are more common in this age group in spite of highly sophisticated equipment. The use of special pediatric blood lines, dialyzers and a higher staff-to-patient ratio make the cost of hemodialysis treatments more for the child than for the adult. Children on intermittent hemodialysis do not resume normal body growth, must adhere to rigid dietary restrictions and suffer the physical and mental distress associated with the thrice weekly insertion of large bore needles.

In-center hemodialysis obligates the family to three trips weekly to the center, demands a redistribution of lifestyle priorities and is generally disruptive to the family unit. Some children are able to attend school part time but many drop out of school. In general, hemodialysis is best suited for older children and adolescents for whom a living related kidney donor will be readily available.

Utilizing the lining of the abdominal cavity as the dialyzing membrane, peritoneal dialysis offers several distinct advantages over hemodialysis for young children. Since there is no need for an extra corporeal circuit of blood, needle sticks are not required to perform the treatments. The inefficiency of the peritoneal membrane as a dialyzing surface is an advantage for small children in that the symptoms and complications of a rapid dialysis treatment are not encountered. Peritoneal dialysis is readily adaptable to most home situations utilizing either automated cycling equipment or continuous ambulatory peritoneal dialysis.

Dialysis treatments can be performed overnight, thus allowing the child unrestricted daytime activities. If receiving treatments intermittently, children on peritoneal dialysis must still adhere to rigid dietary restrictions and the usual metabolic complications of dialysis treatments still prevail.

Long-dwell dialysis therapy, CAPD or CCPD, obviate the need for cumbersome and expensive equipment, maintains a more physiologic but not normal metabolic status and allows most children to forego the customary dietary and fluid restrictions.

Experience with CAPD in children is still limited, however, reports of improved growth and overall well-being are encouraging. Intraabdominal infection, peritonitis, and problems related to the dialysis access, peritoneal catheter, are still prevalent but usually managed with little morbidity or mortality. Peritoneal dialysis does not preclude successful transplantation and is the dialytic modality best suited for young children.

Despite significant advances in dialysis technology, successful renal transplantation remains the optimal form of therapy for children with end-stage renal disease. Physiologic and rehabilitative improvements result only after restoration of normal, or nearly normal, renal function. The initial skepticism of transplanting children proved invalid as successful graft outcome and low patient mortality have been achieved in children 2 years of age and older. Sporadic success has been achieved in children less than 2 years of

age, however, ethical considerations and technical difficulties preclude a general recommendation at this time that transplantation be pursued in children of this age and size. Kidney donation from a sibling over 18 years of age or from a healthy parent is ideal, however, in the absence of an available donor, cadaveric transplantation is quite acceptable.

Following successful transplantation children experience a sense of well-being not previously enjoyed, often achieve a rapid catchup in somatic and intellectual growth, and are effectively rehabilitated for their entrance into adult life.

End-stage renal disease is an expensive illness. It is costly to both medicare in the amount of dollars spent annually to provide treatment and to the child who is experiencing a significant interruption in his or her obtainment of adult life. A multidisciplined approach to therapy utilizing individuals skilled in caring for children with renal failure permits the maintenance of life while undergoing dialytic therapy and a hope for successful rehabilitation following transplantation.

Since congenital disorders account for the majority of children with end-stage renal disease, efforts are currently directed toward earlier identification of renal disease and improved methods of medical management of the child with chronic renal failure prior to the development of ESRD. The more frequent use of abdominal ultrasound in pregnant women offers the opportunity to identify prenatally the infant with obstructive uropathy and thus initiate immediate surgical intervention.

Improved understanding of nutritional requirements in chronic renal failure may change the dietary prescription for these children and improve their otherwise suboptimal growth. Efforts directed toward a better understanding of peritoneal membrane kinetics and the development of techniques to reduce the incidence of peritonitis, should encourage a broader acceptance of peritoneal dialysis in those who are skeptical at the present time.

Improvements in tissue typing and immunosuppressive techniques should improve on the already quite acceptable success of renal transplantation.

Thank you.

[The prepared statement of Dr. Mentser follows:]

PREPARED STATEMENT OF MARK I. MENTSER, M.D., ASSISTANT PROFESSOR OF PEDIATRICS, COLLEGE OF MEDICINE, OHIO STATE UNIVERSITY AND DIRECTOR, RENAL DIALYSIS. CHILDREN'S HOSPITAL, COLUMBUS, OHIO

As a Board Certified Pediatric Nephrologist specializing in the care of children I welcome this opportunity to present to the Subcommittee on Investigations and Oversight my views concerning current and future treatments of children with kidney disease. Recognizing that children and adults share many of the same disease processes and therapeutic modalities, I will direct my comments towards the critical differences that separate the child from the "patient" with kidney disease. Children are different in their metabolic response to disease, have unique emotional needs and require a more individualized method of care.

Overview of Pediatric Renal Disease

The annual incidence of end stage renal disease (ESRD)in the pediatric age group is 1 to 3 new patients/1,000,000 population/year; accounting for approximately 5% of the medicare ESRD population. Congenital renal disorders, obstructive uropathy and renal dysplasia, are the major causes of end stage renal disease in children; whereas, acquired glomerulopathies, hypertension and diabetes are the primary diagnoses in adults. Consequently, children must endure the disease process and its inherent complications during the time of rapid body growth, intellectual development and emotional maturation.

Children who are later to develop end stage renal disease are generally diagnosed before their 10th birthday, with most being identified before 5 years of age. Growth failure is a common presenting sign of renal disease in children even at a point in time when renal function is only moderately reduced. Appropriate therapies directed towards the complications of renal failure are rapidly introduced, however, in spite of early intervention, progressive renal failure ensues. Comprehensive care of these children is best carried out by a renal team consisting of physicians, nurses, nutritionists and social workers. Medical therapy consists of early identification and correction of any obstructive

anatomic abnormality, eradication of infection and aggressive medical prevention of bone disease and hypertension. Nutritional guidance is provided to assure maximal caloric intake since inadequate energy utilization has been identified as one of the many factors related to growth failure. Early psychosocial evaluation provides the renal team with an assessment of the intellectual, emotional and financial needs of the family and better prepares all concerned for future crisis intervention. Testing the child's motor and intellectual skills permits the early identification of deficiencies and institution of appropriate therapy. Eventual development of end stage renal disease dictates the institution of more specific therapeutic modalities, i.e., dialysis and transplantation.

Treatment of ESRD: Current Status

The goal of pediatric ESRD treatment is restoration of the normal growth and development process so that the child may develop to a healthy productive adult. Dialysis therapy, hemodialysis or peritoneal dialysis, controls but does not ameliorate the physiologic abnormalities; and only through transplantation is normal renal function restored and the ultimate goal achieved.

Most children undergo dialysis therapy prior to renal transplantation. Technological advances and highly skilled personnel enable small children and even neonates to undergo successful hemodialysis therapy. Treatment related complications including precipitous drops in blood pressure and convulsions are more common in this age group in spite of highly sophisticated equipment. The use of special pediatric blood lines, dialyzers and a higher staff to patient ratio make the cost of hemodialysis treatments more for the child than for the adult. Children on intermittent hemodialysis do not resume normal body growth, must adhere to rigid dietary restrictions and suffer the physical and mental distress associated with the thrice weekly insertion of large bore needles. In-center hemodialysis obligates the family to 3 trips weekly to the center,

demands a redistribution of life style priorities and is generally disruptive to the family unit. Some children are able to attend school part-time but many drop out of school. In general, hemodialysis is best suited for older children and adolescents for whom a living related kidney donor will be readily available.

Utilizing the lining of the abdominal cavity as the dialyzing membrane, peritoneal dialysis offers several distinct advantages over hemodialysis for young children. Since there is no need for an extra corporeal circuit of blood, needle sticks are not required to perform the treatments. The inefficiency of the peritoneal membrane as a dialyzing surface is an advantage for small children in that the symptoms and complications of a rapid dialysis treatment are not encountered. Peritoneal dialysis is readily adaptable to most home situations utilizing either automated cycling equipment or continuous ambulatory peritoneal dialysis (CAPD). Dialysis treatments can be performed overnight, thus allowing the child unrestricted daytime activities. If receiving treatments intermittently, children on peritoneal dialysis must still adhere to rigid dietary restrictions and the usual metabolic complications of dialysis treatments still prevail. Long-dwell dialysis therapy (CAPD or CCPD) obviate the need for cumbersome and expensive equipment, maintains a more physiologic but not normal metabolic status and allows most children to forego the customary dietary and fluid restrictions. Experience with CAPD in children is still limited, however, reports of improved growth and overall well-being are encouraging. Intraabdominal infection (peritonitis) and problems related to the dialysis access (peritoneal catheter) are still prevalent but usually managed with little morbidity or mortality. Peritoneal dialysis does not preclude successful transplantation and is the dialytic modality best suited for young children.

Despite significant advances in dialysis technology, successful renal transplantation remains the optimal form of therapy for children with end stage renal disese. Physiologic and rehabilitative improvements result only after restoration of normal, or nearly normal, renal function. The initial skepticism of transplanting children proved invalid as successful graft outcome and low patient mortality have been achieved in children 2 years of age and older[1,2]. Sporadic success has been achieved in children less that 2 years of age[3,4], however, ethical considerations and technical difficulties preclude a general recommendation at this time that transplantation be pursued in children of this age and size. Kidney donation from a sibling over 18 years of age or from a healthy parent is ideal, however, in the absence of an available donor, cadaveric transplantation is quite acceptable. Following successful transplantation children experience a sense of well being not previously enjoyed, often achieve a rapid catch up in somatic and intellectual growth, and are effectively rehabilitated for their entrance into adult life.

Kidney Disease in Children: Future Goals

End stage renal disease is an expensive illness. It is costly to both Medicare in the amount of dollars spent annually to provide treatment and to the child who is experiencing a significant interruption in his or her obtainment of adult life. A multi-disciplined approach to therapy utilizing individuals skilled in caring for children with renal failure permits the maintenance of life while undergoing dialytic therapy and a hope for successful rehabilitation following transplantation. Since congenital disorders account for the majority of children with end stage renal disease, efforts are currently directed towards earlier identification of renal disease and improved methods of medical management of the child with chronic renal failure prior to the development of ESRD. The more frequent use of abdominal ultrasound in pregnant women offers the

opportunity to identify prenatally the infant with obstructive uropathy and thus initiate immediate surgical intervention. Improved understanding of nutritional requirements in chronic renal failure may change the dietary prescription for these children and improve their otherwise suboptimal growth. Efforts directed towards a better understanding of peritoneal membrane kinetics and the development of techniques to reduce the incidence of peritonitis, should encourage a broader acceptance of peritoneal dialysis in those who are skeptical at the present time. Improvements in tissue typing and immunosuppressive techniques should improve on the already quite acceptable success of renal transplantation.

Conclusions

Medical expertise and technological advances have allowed children to become an integral part of the end stage renal program. Restoration of normal renal function with subsequent rehabilitation is the goal of therapy achieved through successful transplantation. This is the most cost-effective form of treatment in terms of both overall dollars spent throughout the child's life and also in terms of the length of interruption of the child's life style. Prior to transplantation, approximately 30% of children receiving dialysis therapy were undergoing well-tolerated and cost-effective home peritoneal treatments. Efforts are being directed towards improvement of both therapeutic modalities and the earlier identification and treatment of children with kidney disease.

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Mr. GORE. Thank you very much for excellent testimony. Obviously you spent a lot of time on that and I appreciate it.

Our next witness is Dr. Lee Hebert, professor of medicine and director of the division of renal diseases here at Ohio State and we are delighted to welcome you.

You are accompanied by Dr. Ronald Ferguson, correct?

Dr. HEBERT. Yes, that's correct.

Mr. GORE. Who is going to do the testimony?

Dr. HEBERT. Well, there is a background testimony I will give and Dr. Ferguson will speak to the issue of Cyclosporin A, which is very pertinent this morning.

Now, I prepared this presentation much like we present medical information. I have slides. The slides are going to be projected up there, which means you can either break your necks or you can sit over there.

Mr. GORE. We will recognize you and move over there so we can see it.

Dr. HEBERT. I need a pointer.

On behalf of the department of medicine and the renal division of Ohio State University, I would like to thank Mr. Gore and Mr. Shamansky for this opportunity to express our ideas on the important issue of ethical aspects of the care of patients with end-stage renal failure and also to comment on the biomedical research effort, particularly as it relates to renal disease.

In 1965, when I left for Boston to work with Dr. William B. Schwartz, one of the leaders in the fledgling field of nephrology, there was no such thing as long-term dialysis for patients with kidney failure.

In those days, when patients progressed to irreversible kidney failure, we told them, with all the compassion we could muster, that there was nothing further that could be done.

We would then work mightily to show our concern and support and provide them with whatever medications were available to ameliorate their symptoms.

As you might well imagine, these were ghastly experiences, but they got worse.

By 1970 the technology of dialysis improved sufficiently to permit us to keep patients with kidney failure alive for prolonged periods. The agony of this technological advance was that dialysis was not available to all who needed it.

Mainly dialysis was a perquisite of the rich or those with incredibly indugent insurance policies.

To cope with this problem, which was even more agonizing than having no dialysis at all, committees were set up to decide who shall and who shall not receive free dialysis. That is, who we could not afford to keep alive.

I can clearly recall participating in these draconian committees, which were awful, as you can imagine.

As if by miracle, in 1973, this burden was lifted from us when the Federal Government said it would provide most of the means to support dialysis services to patients with kidney failure.

At this point it seemed our problem was solved. We were wrong.

It was originally estimated that about 25 patients per million per year would arrive at end-stage renal failure and most would eventually receive successful kidney transplants. This formula proved grossly incorrect.

As the years went by, 25 to 50 to 75 patients per million and, in some parts of the country such as Florida with a high elderly population, 300 patients per million were arriving each year at end-stage kidney failure.

Patients who previously had not even considered dialysis because of cost, age, debility, remote location or other difficulties, were now being rapidly provided with one form of dialysis or another.

In addition, our patients began living longer, as we got smarter.

First slide, please.

This shows the OSU experience.

This shows the survival of our patients on dialysis as a function of time, and I simply point out in the first 4 years there is about an 8-percent per year mortality rate.

That would be very respectable among nationally available data, and I think is even more impressive when you realize that the dialysis population at the Ohio State University is heavily transplanted because of the aggressive department headed up by Dr. Cerilli up until just recently and now headed up very capably by Dr. Ferguson.

So that's what happens to the patient.

They live much longer. It used to be like 30, 50 percent 1-year mortalities.

Next slide.

The final factor that led to the burgeoning number of patients on dialysis is that transplantation didn't keep pace with the large number of patients coming onto dialysis, and the reasons for this are complex and we will address that later on. I assume some of the questions will come up about that and we will talk about that some more.

At any rate, we are now left with a large number of patients who are old, somewhat debilitated and must be regarded as having life of low quality. A recent review of this topic—next slide, please.

The New England Journal of Medicine, I think it was probably 1981—this shows the activity level of patients on dialysis. And, as you can see in this particular group of patients, roughly 60 percent of the patients had a good quality of life.

Twenty percent were able only to care for themselves. For diabetics, the situation was much worse.

Thanks to Linda and Renee and Sherry this morning, we have our own data and they are remarkably similar, including diabetics and nondiabetics.

We found 61 percent in this category and 20 percent in this category or less and we had no more, so this is quite typical and reflects our own experience.

Again, this is a heavily transplanted population, so you intend to transplant the healthy patients. The sicker patients remain on dialysis, so I think we speak well of our dialysis personnel.

The question as to whether the heroic and very expensive therapy of chronic dialysis is appropriate in such patients must be asked.

To answer that question, we can begin by asking the patient whether he or she wishes to receive dialysis therapy.

There is no firm data on this but I can tell you from my own experience which now spans approximately 16 years and well over a thousand patients with end-stage kidney failure, that it is rare for a patient to refuse lifesaving dialysis.

Nevertheless, in a world where two-thirds of the people are said to go to bed hungry each night, how can we justify spending $25,000 a year or more to maintain a debilitated individual who simply wishes not to die and contributes nothing to society and so on?

However, before we become too scandalized, we recall that it costs the Government anywhere from $10,000 to $25,000 a year to sustain a convicted felon in prison.

Clearly the dialysis patient, whose only sin against society is that his kidneys happened to have failed, has as much right to life as the convicted felon. In fact, there are a lot more convicted felons than dialysis patients.

However, it is not sufficient for me to simply state the dilemmas. I must propose rational solutions.

Next slide, please.

These are short-term remedies. These clearly work and we want to focus on this perhaps in a discussion period.

We are encouraging transplantation, performance standards. There are too many dialysis centers in this country which are grossly inadequate, but there is very little effort to those outside their own areas to provide the patients with transplantation and, to some extent the networks—like over this year, there is too much—and Dr. Cerilli who was chairman of our network knew of the good-old-boy system, and I personally believe there should be national performance standards that are applied to dialysis; make sure that they do everything they can to transplant the patients they can.

A second issue, we should discourage the proliferation of chronic incenter dialysis units. I think again, Dr. Bay, Dr. Cerilli will probably address this issue.

This is a common phenomenon of a small community of three or four or five patients wanting to have its own dialysis center so that patients don't have to travel so far to receive dialysis therapy for those who are not candidates for home dialysis. And this is a very commendable thing.

Unfortunately, it is a very inefficient system because what happens is these small dialysis centers in general—there are many exceptions, obviously—but in general they tend to be not all integrated into the major areas and these patients tend not to move into the activities of the transplant centers and thus there is not efficient movement of patients with end-stage renal failure into transplantation or alternate modes of therapy such as home dialyasis.

In other words, by creating lots of dialysis programs in small communities all over the place.

Obviously, however, these remedies do not solve the problem. What is needed is the means to prevent a patient from progressing to end-stage renal failure, better and cheaper means to manage the patient with renal failure by dialysis or perhaps nutritional techniques and, finally, strategics to provide successful kidney transplants to more patients.

Dr. Ferguson will address that issue with respect to Cyclosporin A.

The means to achieve these goals are shown on the next slide.

Obviously there is an element of humor here, but I am dead serious.

As you know, research will provide the answer and I can only tell you, you know, when I graduated from medical school in 1962, if we were to compare a sophisticated hospital in 1962 to a sophisticated hospitals in the 1980's, in comparison with what we can do for patients, it is incredible.

It is Star Wars all over again.

When I was an intern 100 million years ago, the most sophisticated piece of equipment was called a bird respirator. And if you were in a hospital that had two bird respirators, you were a medical center. And all those did was blow air in your lungs. Now it's just Star Wars and we have solved so many things. There is no question.

A discussion of specific research directions is not practical in today's restricted format. However, you might find it useful to review the range of research activities of the Ohio State University Renal Division. I didn't include the transplant division, but that would be substantial.

Perhaps Dr. Ferguson will address that.

But anyway, what we do hope is quite typical of what is going on in most major academic medical centers.

Next slide, please.

I think some of these points are relevant. This is what is being done. This is the renal division. This is, of course, our major project.

This is—you can see the source of funding and so on, duration of the study, clinical studies in hypertension, Dr. Bay, the Revlon Corp.

But the spinoff, excess money is used to provide support for a nurse investigator for these studies. This is all going very nicely.

We are involved in two national collaborative trials. This one and that one are the only two. We get a small amount from the NIH. This doesn't nearly cover the cost.

They are enormously consuming of time and effort and so on, but well worthwhile. I think we are the larger contributor to this trial and the second largest contributor to this trial.

Finally, the newest member of our group is involved in this. I want to call this to your attention.

This is very typical in academic centers where the academic programs will put their own funds into taking patient care into their own research. We believe that is what we are doing.

I was trying to get your sympathy. One of my reasons will be—slide off, please. Slide on. Excuse me.

Rhetorical question: Is this research important? What if this turns into a big bust? I hope not, but what if plasmapheresis is ineffective? What if the INS trial shows that they are no good?

When research fails to produce the breakthrough—and it must be acknowledged that most research projects do not produce breakthroughs—does that mean the time, effort and money expended has been wasted?

Does this mean that most research projects are a waste? An expensive luxury that can be tolerated in good economic times but should be sacrificed in difficult times?

I respectfully submit to you that the answer to each of these questions is a resounding no.

Research has enormous practical value, with or without the breakthrough.

At the risk of belaboring the obvious, may I briefly present an allegory demonstrating this point?

Next slide, please.

I brought this to the artist here and we got a very nice thing put together by the artist but it was wrong, so I had to do it by hand yesterday, and that's why it looks like this.

Anyway, these people are building a knowledge structure. This knowledge structure could be addressed to investigating any discipline, how to best fertilize a field and so on.

The point is that because these people are investigators, it is crucial to their survival as investigators that they efficiently build this knowledge structure as tall and as strong as possible.

To do that, each new piece of information which may have been generated by themselves or others, must be precisely fitted into the preexisting knowledge structure and the previously assembled piece of information must be carefully maintained.

Thus these investigators have the pressing need to seek as much new information as possible, to integrate that information as precisely as possible and to maintain it impeccably. Their survival as investigators depends upon it.

The value of this fine knowledge structure to their research program is obvious. However, it is also obvious that this knowledge

structure will be extremely valuable in their teaching responsibility and, if these are medical investigators, enormously valuable in their tertiary level care of patients.

Now, I would like you to consider what kind of knowledge structure can be built in research if research is not being done; that is, if one does not require a knowledge structure capable of being tested to the limit.

All that is needed is a decent knowledge structure sufficient to get the job done.

Next slide, please.

I respectfully submit to you that this is the likely outcome. There is no question that a knowledge structure is being built, however, not very tall. There are gaps in it, but it is sufficient to get by and that is all that is necessary.

Consider, however, that this person, not working with the benefit of a research environment, is responsible for teaching, that is, imparting the concepts of the knowledge structure to his students.

How clearly and precisely do you think the concepts of that knowledge structure will be in the minds of the students?

My simple point is that the fact of doing research is extremely important because it creates the pressing obligation to build excellent knowledge structures.

These knowledge structures are of great practical importance, in good economic times and in bad.

This, in a sense, is good news. Research is valuable. The bad news is that the biomedical research community is in crisis. The reason for the crisis is that the life blood of the research community, the Federal research support, is slowly being withdrawn.

The increase in total at the NIH barely keeps up with inflation, and in its renal stage it has done a little better, but other areas are dying.

The effects of exsanguination are not legal as yet by the signs of serious weakening are obvious.

Currently only 15 to 25 percent of NIH grants are being funded. This means that only 15 to 25 percent of the best ideas of our best investigators are able to receive funding.

This fierce competition for grants is changing the pace and direction of research. research projects which may be quite important but may require a long period of time to develop are being avoided in favor of research projects which are likely to yield quick punishable results. This is done to show evidence of continued high level productivity, a necessity to successful grantsmanship.

As a corollary, there is increased tendency to seek research support from private industry for projects of commercial value. Although this clearly has desirable aspects, it must be borne in mind that if private industry controls the tempo and direction of research, this may not permit the development of the theoretical bases needed for effective long-term solutions.

For example, if private industry were the dominant force in biomedical investigation in renal disease, a plausible outcome would be the development of many different kinds of catheters for dialysis but not the cure for renal disease. This is not a criticism of private industry.

Rather, it acknowledges that the goal of private industry and the goals of academic medical investigation can be strikingly different. Clearly both are needed but one cannot substitute for the other.

Perhaps the most ominous trend is that established investigators are becoming disheartened and are leaving their careers in academic medicine for private practice or industry and new investigators are being discouraged from embarking on careers in investigation.

Biomedical investigation is becoming an undesirable career.

Medical students no longer want to emulate their professors. This is largely because of the uncertainty of success and fulfillment in this field, which so strongly derives its fulfillment from the ability to perform effective research.

Because of this, the biomedical fields of investigation will no longer attract top students. Instead, these students will choose careers in nonmedical fields or will choose to become clinicians with the greater security and financial rewards of private practice.

If this should occur, we can look forward to the gradual deterioration of our academic medical centers until they will function as community hospitals.

This, of course, is not a criticism of community hospitals which play a vitally important role in overall patient care. However, it must be clearly borne in mind that virtually all the advances in medical science occur in academic medical centers, not in community hospitals.

Without a vigorous research community at the core of the medical center, the physician can never be more than a practitioner, a conduit of preexisting information.

Furthermore, when the practitioner as teacher attempts to impart his knowledge and skills to students of medicine, there is likely to be further erosion of knowledge since there is nothing within the system to renew and expand his knowledge.

This obviously has the potential of becoming a vicious cycle with the outcome being a progressive decline in the effectiveness of the health sciences and an enormous social and economic cost to society.

In closing, I sincerely believe what I have just told you is not hysterical rhetoric, but is inexorably becoming fact. I talk very fast. I am not hysterical, but it is.

I believe that adequate support of the biomedical investigation should be one of our country's highest priorities.

I believe we must ask ourselves whether it is worth critically debilitating the biomedical investigative community to have another 5,000 foot soldiers or another dozen tanks.

I respectfully submit to you that in this crisis the health of this Nation and of the world is at stake since, as you know, America is the undisputed leader in health research.

Thank you for your interest.

I sincerely hope that I have not failed in my task to impart to you the critical importance of saving the biomedical research community.

Thank you very much.

[The slides used during the presentation, plus the prepared statement of Dr. Hebert follow:]

LONG-TERM SOLUTION TO THE PROBLEM OF EXCESSIVE NUMBERS OF PATIENTS WITH END-STAGE RENAL FAILURE

1. RESEARCH

2. MORE RESEARCH

Table 3. Physical Activity of 2191 Nondiabetic and 289 Diabetic Dialysis Patients, According to Karnofsky Score.*

KARNOFSKY SCORE	NONDIABETICS	DIABETICS
	per cent of group †	
90–100 (almost normal)	33	8
80–89 (active)	27	15
70–79 (self-care only)	21	25
40–69 (debilitated)	18	47
1–39 (moribund)	2	4

*The general difference between nondiabetics and diabetics is highly significant ($P<0.001$).

†Values do not total 100 per cent because of rounding off.

CURRENT RESEARCH AND RESEARCH SUPPORT
OF THE OHIO STATE UNIVERSITY
RENAL DIVISION
DRS. BAY, COSIO, HEBERT, ZAGER, WAXMAN

	AMOUNT	SOURCE	DURATION
IMMUNE COMPLEX MEDIATED GLOMERULONEPHRITIS	375,000	NIH	1981-85
FACTORS WHICH MODULATE RENAL TUBULAR INJURY	32,000	KIDNEY FDN, HEART ASSN, OSU	1981-83
CLINICAL STUDIES IN HYPERTENSION	26,000	REVLON CORP	1982
CLINICAL STUDIES IN CONTINUOUS AMBULATORY PERITONEAL DIALYSIS	NONE	--	ONGOING
PLASMAPHERESIS IN SEVERE LUPUS GLOMERULONEPHRITIS (NATIONAL COLLABORATIVE TRIAL	4,000	NIH	1982-85
THERAPY OF ADULT GLOMERULAR DISEASES (NATIONAL COLLABORATIVE TRIAL)	NONE	--	1979-85
IMMUNE COMPLEX BINDING AND METABOLISM BY HUMAN LEUKOCYTES	18,000	KIDNEY FDN, OSU	1982-84

FUNDS CONTRIBUTED FROM RENAL DIVISION PRACTICE FUNDS WHICH ARE USED TO SUPPORT RESEARCH: APPROXIMATELY $92,000 PER YEAR.

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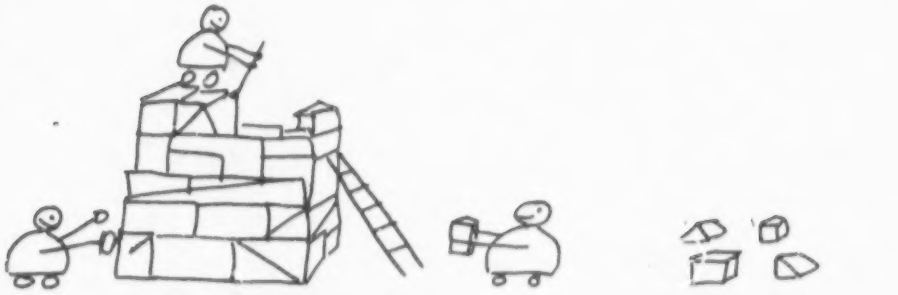
ACTIVE RESEARCH ENVIRONMENT



INACTIVE RESEARCH ENVIRONMENT

SHORT-TERM REMEDIES TO THE LARGE NUMBER OF PATIENTS REQUIRING DIALYSIS SERVICES

1. ENCOURAGE RENAL TRANSPLANTATION.

2. DISCOURAGE THE PROLIFERATION OF CHRONIC IN-CENTER DIALYSIS UNITS.

October 6, 1982

ANALYSIS OF THE CURRENT STATUS OF BIOMEDICAL INVESTIGATION PARTICULARLY AS IT RELATES TO RENAL DISEASES

Lee A. Hebert, MD
Professor of Medicine
Director, Renal Division
The Ohio State University Hospitals

During the past fiscal year, over 1 billion dollars was spent by the Federal government to care for slightly more than 50,000 patients with end-stage renal disease (ESRD), who require therapy by dialysis or renal transplantation. The rapidly rising cost for this program was not anticipated when, in 1973, Congress enacted legislation which provided coverage for 80% of the cost of therapy for end-stage renal disease to individuals who are eligible for Social Security benefits. Because of the mounting costs to the Federal government for ESRD services for a relatively small number of patients, intensive efforts are underway to identify strategies to control costs for ESRD services. The principal strategies are as follows:

1. Reduce reimbursement to providers of ESRD services

2. Limit the number of dollars available for ESRD services (in effect, limiting the number of patients who can receive treatment for ESRD)

3. Intensify research to discover means to:

   a. Prevent ESRD or slow progression to ESRD

   b. Manage dialysis therapy more cost-effectively

   c. Provide successful kidney transplants to more patients.

The first strategy has been acted upon and seems appropriate. The second strategy involves enormous ethical dilemmas and has largely been ignored. The third strategy, which provides the only long-term effective solution, has received little attention. My purpose is to comment on the desirability and necessity of carrying out the "research strategy" and to discuss the consequences if we fail to carry out the research strategy.

Currently the biomedical research community is in crisis. The reason for the crisis is that the lifeblood of the research community, Federal research support, is slowly being withdrawn. The effects of this slow exsanguination are not fatal as yet but the signs of a serious weakening of the research community are obvious. The principal signs are as follows:

1. The number of research grants funded by the NIH is falling steadily. Currently, only 15 to 25 percent of the best ideas of our best biomedical investigators are able to receive Federal funding.

2. The competition for funding of grants is fierce. For many investigators, the time spent preparing the grant application to produce a grant proposal which is not only scientifically sound but also socially, economically, politically and cosmetically attractive, is seriously erroding into the time that can be spent doing research.

3. There is an increased tendency in the scientific community to avoid sharing new research insights for fear that valuable (fundable) ideas may be adopted and developed by others. Thus, the most effective catalyst to the generation of new research directions, the spirited and open exchange of ideas among investigators, is on the decline.

4. Important research projects that may require a long time to develop are being rejected in favor of projects that are likely to yield quick, publishable results. This is done to show evidence of continued high-level productivity, a necessity in successful "grantsmanship". Clearly, this is not in the best long-term interest of scientific medicine. As a corollary, there is an increasing tendency to seek research support from private industry for projects which have commercial value. Although this clearly has desirable aspects, it must be borne in mind that if private industry controls the tempo and direction of research this may not permit the development of the theoretical bases for long-term solutions. For example, if private industry were the dominant force in biomedical investigation in renal disease, a plausible outcome might be the development of many different kinds of catheters for dialysis, but not the cure for renal disease. This is not a criticism for private industry. Rather, it acknowledges that the goals of private industry and the goals of academic biomedical investigation can be strikingly different. Clearly both are needed and one cannot substitute for the other.

5. Established investigators are becoming disheartened and new investigators are being discouraged from embarking on careers in investigation. Perhaps this is the most ominous trend of all since it appears that academic biomedical investigators are headed for extinction or, at best, such greatly reduced numbers that they will be ineffective in the further development of the biomedical sciences. Moreover, since biomedical investigation is becoming such an undesirable career, because of its uncertainty and lack of fulfillment, the biomedical academic sciences will no longer attract those with the highest skills. Instead, those who are most enterprising will choose careers in nonmedical fields or will choose to become clinicians with the greater security and financial rewards of private practice. If this should occur, we can look forward to the gradual deterioration of our academic Medical Centers until they will simply be community hospitals. This should not be viewed as a criticism of community hospitals which, of course, play a vitally important role in overall patient care. However, it must be clearly borne in mind that virtually all of the advances in medical science occur at academic Medical Centers, not in community hospitals. Moreover, the impetus for advances in medical care come from the strong research community within the academic Medical Centers.

> Without a vigorous medical community at the core of the Medical Center the physician can never be more than a practitioner, a conduit for pre-existing information. Furthermore, when the practitioner as teacher attempts to impart his knowledge and skills to the student of medicine, there is likely to be a further erosion of knowledge since there is nothing within the system to renew and expand his knowledge. This obviously has the potential of becoming a vicious cycle with the outcome being a progressive decline in the effectiveness of the health sciences and enormous social and economic costs to society.

In summary, the community of biomedical investigators almost certainly possesses the ability to solve the problem of end-stage renal failure. However, the resources available to carry out that project are grossly inadequate. Indeed, because of the deprivations within biomedical investigation, it is becoming unattractive as a career choice for young investigators and established investigators are leaving the field for the greater security and material rewards of the private practice of medicine or private industry. If this trend continues, there will be enormous social and economic costs to society for our failure to properly support the biomedical investigative community. I believe that adequate support of biomedical investigation should be one of our Country's highest priorities. If we delay in providing that support, it will take decades to repair the damage which will have been done.

Mr. GORE. Thank you very much, Doctor. I really learned something from that and I appreciate it.

Let me call now on Dr. Ferguson.

Dr. FERGUSON. I did not know I was going right now. I have four slides, if you would not mind.

Mr. GORE. And we need to make the slides available to the reporter, also.

Dr. FERGUSON. Perhaps first I should introduce myself a bit. I am the newest member of the team here at the medical center that cares for end-stage renal disease patients and have assumed the role of directing the transplant—renal transplant—at the Ohio State University very recently after Dr. Cerilli's departure.

I came from the University of Minnesota. And what I would like to do now is reiterate and reemphasize a few points that were made earlier and supplement it with a specific example and some data on cost effectiveness in renal transplantation and perhaps something about the impact of Cyclosporin A, which is a new immunosuppressive therapy and, in particular, how that may impact on the cost of renal transplants.

Can everybody hear me now?

To reiterate, I think the emphasis was placed today on research of the prevention of end-stage renal disease, but it is not here yet.

And until the implication of those things yet to come are in mind, which is really what would be the cost-effective preventing of renal disease, what do we do in the interim? How can we most efficiently and most cost effectively apply the technologies that are available today to the end-stage renal disease patients?

Something I would like to reemphasize is what Dr. Cerilli went through. One is the cost of the transplantation. It is truly the least expensive of the therapeutic modalities for end-stage renal disease. The limited application of it—and perhaps to answer, in part, your question, Congressman Gore, of earlier, the reason so many—one of

the reasons not so many transplants are performed is because of that traditional 55 percent 1-year cadaveric graft function. And not only for graft survival, but there are very crude endpoints.

What about patient morbidity on the way? Rehospitalization, complications, costs incurred during these?

I think things have changed. Transplantation is young. And in the last 2 years in particular, there have been major strides, both as Dr. Cerilli mentioned and as Dr. Salans mentioned; you can see many related transplants and cadaveric transplants, particularly with Cyclosporin A.

And one point I really want to make clear to you is that 55 percent cadaveric graft function 2 years from now is not going to be the standard norm. It is going to be probably in the neighborhood of 80 percent. That is considerably different. So if one has those kinds of end results for patient survival greater than 90 percent—usually 95 percent—what other endpoints in the field of renal transplant can we look at?

Well, obviously, patient morbidity, hospital readmission, hospital dates and costs become paramount in comparing anything and in looking at the particular application of this technology.

Throughout the past 2 years at the University of Minnesota, I was directing a study that was looking at a comparison of Cyclosporins A with conventional immunosuppression in cadaver and other related transplants. In over 100 and—well, there were about 170 patients entered in that study over the 2-year period. And the results were pretty comparable.

We were getting 85 percent, 22 on the graft function with first cadaver kidneys. We were getting 80 percent graft function with second cadaver kidneys, and we were getting about 90 percent graft function with mismatched and unrelated donor kidneys.

But the results that Dr. Cerilli had mentioned earlier, these should be what is expected on a national level 3 years from now. When one is looking at those kinds of results, what you look at to compare, if you have these to compare to, which is better?

One of the things we looked at is rejection. Fifty-seven percent of the patients who took the conventional therapy were admitted to the hospital. Seventeen percent Cyclosporin patients. There were fewer than 50 percent—there were fewer than half of the infectious complications after transplantation.

It gave equal results, fewer rejections, fewer infections. How did this translate into cost and hospital base, which is the bottom line that I would like to show you now.

Can I have that first slide, please?

This is 67 patients treated with conventional therapy, 70 treated with Cyclosporin, all followed at least 6 months.

This is the readmission rate, percent never readmitted, the total number of readmission, the number of patients and the number of—per patient—of readmissions after transplantation.

You can see that half the patients treated with Cyclosporin are readmitted. And I must admit this represents a learning curve, too, so it is probably less now.

Three-quarters of the conventional therapy patients were—this is a highly significant difference. How does this translate into a little more detail?

Next slide, please.

Hospital dates. This is initial hospitalization when they got their transplant, the first readmission and subsequent readmissions, remembering that fewer patients were readmitted. This was not quite cut in half but substantially reduced, highly significant. And then in particular, subsequent admissions were much less, so that if one calculates the total readmissions, there is a highly significant difference in the number of hospital readmission days per patient. This is, while achieving, if you will, the same endpoints of patient graft survival.

Next slide, please.

How does this translate into cost? I went back and looked at 40 consecutive Cyclosporin and 40 consecutive conventional therapy patients, all who had been followed 6 months, went and pulled their hospital bills and found out how much they were billed and what was paid. And what we found in the initial hospitalization—and I will show you the specific numbers—was there was a 38 percent reduction in initial hospital days with a 27-percent reduction in cost. There was a 50-percent reduction in readmission rate with a 59-percent reduction on the cost on readmission when one compares the two groups of patients.

Can I have the next slide, please?

Dr. Cerilli told you the approximate cost of a transplant. These are actual numbers of what it cost 40 consecutive conventional therapy and 40 consecutive Cyclosporin patients. The initial hospitalization, if you look down here before you look up here, the initial hospitalization, this was the mean for Cyclosporin. This was the mean for conventional therapy. Percent of patients first readmitted at this unit cost per readmission, so it was much less expensive for Cyclosporin. Percent subsequent readmissions at this cost. These are actual numbers.

Now, if one projects readmission rates, initial hospital days and uses these 80 patients as a basis to compare and project per hundred transplants, one gets these figures up here. And this is per hundred Cyclosporin patients versus per hundred conventional therapy patients. It is, over the first 6 months, approximately $1.6 million per hundred in savings in hospital costs.

So that not only can you get what results with end points in graft survival, you can decrease patient morbidity and save considerable amounts of money—if one uses this new particular immunosuppressant.

I think it's particularly appropriate to say, also in the context of Dr. Hebert's discussion, because it is a specific example of what can happen; clearly, the cheapest way to do things is to do them badly.

How do you do them better? You find ways to do them better, and that is research. Research, using the application of this particular new immunosuppressive agent, its application has increased patient graft—or decreased the morbidity, maintained very good patient graft survival, and it does it far cheaper.

So I think it points out a number of issues and a specific example of that which was brought up this morning.

[The slides used during presentation follow:]

RANDOMIZED PROSPECTIVE TRIAL
FREQUENCY OF READMISSION AFTER TRANSPLANT
CYCLOSPORINE vs CONVENTIONAL THERAPY

	N	% READMISSION*	% NEVER READMITTED	TOTAL READMISSIONS # ADMIT	# PATIENTS	#/PATIENT
CONVENTIONAL	67	73% (49)	27% (18)	80	49	1.2
CYCLOSPORINE	70	50% (35)	50% (35)	53	35	0.75

* $p < .01$

RANDOMIZED PROSPECTIVE TRIAL
ANALYSIS OF HOSPITAL DAYS
CYCLOSPORINE vs CONVENTIONAL THERAPY

	INITIAL HOSPITALIZATION		FIRST READMISSION		SUBSEQUENT READMISSION		TOTAL READMISSION	
	MEAN	TOTAL	MEAN	TOTAL	MEAN	TOTAL	MEAN	TOTAL
CONVENTIONAL	21.3 ± 19	1420 (67)	14.3 ± 10	725 (49)	13.2 ± 10	410 (31)	14.2 ± 11	1135 (80)
CYCLOSPORINE	12.6 ± 7	858 (70)	12.7 ± 12	444 (35)	5.7 ± 4	103 (18)	9.9 ± 5	547 (53)
	$p < .001$		NS		$p < .01$		$p < .01$	

( ) = NUMBER OF ADMISSIONS

PROSPECTIVE RANDOMIZED TRIAL
SUMMARY OF COMPARATIVE
HOSPITAL DAYS AND COST

40 CYCLOSPORINE AND 40 CONVENTIONAL THERAPY PATIENTS
ALL FOLLOWED >6 MONTHS

INITIAL HOSPITALIZATION WITH CYCLOSPORINE	READMISSION
38% REDUCTION IN HOSPITAL DAYS	52% REDUCTION IN READMISSIONS
27% REDUCTION IN HOSPITAL COST	59% REDUCTION IN COST PER READMISSION

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PROJECTED COMPARATIVE TOTAL COST OF 100 RENAL TRANSPLANTS: CYCLOSPORINE vs CONVENTIONAL THERAPY*

	100 Cyclosporine	100 Conventional
Initial Hospitalization	$ 2,437,500	$ 3,350,100
First Readmission	199,550	769,144
Subsequent Readmission	221,550	393,617
	2,858,518	4,512,861
Total Savings	$ 1,654,343	

* Based On: 40 Conventional and 40 Cyclosporine Patients All Followed at Least 6 Months

Initial Hospitalization Unit Cost:	$ 24,375	$ 33,501
% First Readmission	50%	72%
Unit Cost:	3,991	9,736
% Subsequent Admission	26%	49%
Unit Cost:	8,518	8,033

Mr. GORE. Thank you very much for that fine testimony. We appreciate it.

Our final witness on this panel, and our final witness of the day, is Dr. Samuel Gorovitz, professor of philosophy at the Department of Philosophy at the University of Maryland at College Park, Md. And, Dr. Gorovitz, I appreciated the opportunity to visit with you earlier and appreciate the work that you have done in the bioethics area, and we welcome your testimony today.

Dr. GOROVITZ. Thank you. I am very pleased to have been included among those who have an opportunity to present views.

My perspective as a philosopher rather than as a physician is going to be quite different from those we have heard so far today. I don't have a previously prepared statement; I do have a number of reactions to the issues as I have thought about them and as my reflection has been prompted by the information introduced today. I am going to say some things about kidney disease, but I want to approach them by what might at first seem a somewhat more circuitous route than the direct approach we have heard so far.

I want to reflect a bit about health care as a general social phenomenon before talking about kidney problems specifically. I notice that it is a phenomenon for which there is not, and really cannot be, a free market in quite the same way in which there can be for CB radios and color television sets. The reasons for that are many and complex.

One factor is that it is not possible to saturate the market. There is no such thing as sufficiency of health care. It is unlike television sets, CB radios, and the like. The better it gets and the better and more extensive its distribution, the greater the ultimate demand for it. One of the consequences of successful treatment of end-stage renal disease patients is that they survive to become the recipients of health care for other ailments. They survive to become the recipients of continuing health care for their kidney problems. The patient who does not survive ceases to be a health care consumer. The more effective his health care, particularly in the face of critical illness, the more longevity he will have as a source of demand for health care.

Now, health care has changed astonishingly in recent times. For most of the history of the provision of medicine and medical treatment, it could not do much. It was mostly provision of comfort, and, perhaps, of confidence and hope which could have direct therapeutic benefits over a limited range. That is about all. It was more aspiration than accomplishment. In recent times, medical care has become tremendously powerful in its capacities to achieve the objectives that it aspires to achieve.

It is useful to think of medical care now as being in its adolescence, in a very specific way. I think adolescents are marvelous. They are fascinating people. Perhaps the most significant characteristic of adolescents is the rapid acquisition of a broad range of new capacities and powers. Physical powers, intellectual powers, sexual, and psychological development all come on in a rush. And associated with that is the stress and anxiety that results from the rapid acquisition of those powers without the wisdom that comes through time and maturity about how to use and exercise those powers.

That seems to me to be precisely the circumstance that medical care is in right now. That is, there has been a rapid and dazzling acquisition of powers, so that now medical intervention not only can achieve much of what it once aspired futilely to achieve, but has capacities that go beyond those aspirations.

Medical intervention can now do more than it is reasonable for it to aspire to do in some cases. The standard example that illustrates that point is the capacity to keep alive someone who is irredeemably dysfunctional, who perhaps has minimal cognitive functioning, who is a physical wreck, the mere shadow or hulk of a former person, but who can be kept quasi-alive by contemporary medical intervention—perhaps for decades—at enormous cost.

So medicine, like adolescents, I see as embodying marvelous powers without the accumulated wisdom that we call maturity, not through any fault of its own, but because it all happens too fast and in too much isolation from a broader societal reflection about what kind of wisdom should accompany medical intervention.

The dialysis program provides an important case in point, and it illustrates a broader problem which will not disappear even if the dialysis problem is resolved. When I refer to the dialysis problem, I refer to the very large cost. In one sense, $2 billion a year falls well within the range of dubious governmental expenditures. On the other hand, it is a lot of money, especially when we recognize that there are other diseases which, though less fashionable, are no less devastating, and there can be a growing advocacy of comparable treatment for the sufferers of diseases for which there are not presently comparable Federal programs.

Now, when I speak of the dialysis problem being solved, I take it that the problem at present is there is a cure for a large category of patients, but it is expensive. It is expensive to a large extent because of the breadth of that category.

Ways of solving such a problem are reducing the number of patients through prevention or reducing the cost through more effective and cheaper methods of cure. I agree with everything that has been said in advocacy of basic research—as well as very practically oriented research—for its own sake and not merely for the immediate benefit that we can expect. But there will always be new state-of-the-art medical interventions so long as we continue to have research, and the associated problems of cost and of distribution won't go away.

One of the items in the news just this week is the NMR scanner, which is, in many respects, better than the now famous CAT scanner. The NMR scanner eliminates radiation problems; it gives a finer discrimination; it is less intrusive. There are about a dozen of them functioning in the country today. It is estimated that in a year there will be 50.They cost about $1.2 million apiece. When every rural medical center wants its own NMR scanning equipment, we are going to face a very substantial question about Federal support and other kinds of support for equal distribution throughout the society of state-of-the-art medical care.

People now are at work on implantable artificial hearts—the potential in economic terms for the success of that research program is staggering to contemplate.

What that says to me is that the problem of renal dialysis and associated problems of transplant provide one case study of a much broader category that it is most useful to think of as the category of tragic choices. When I refer to tragic choices, what I mean specifically is those situations in which we are forced to choose among values that we hold dear societally, none of which we wish to sacrifice in deference to another.

The satisfaction of medical needs, on the one hand, versus our respect for the liberty of individual practitioners, or the other, is one kind of conflict of that sort. We can equalize distribution by limiting the liberty of medical practitioners, but that is a conflict that confronts us with a tragic choice.

The tragic choice that is perhaps most related to the question of renal treatment is that of equity and fairness versus efficiency. We want to limit costs, but we don't want to limit treatment. And we know that fairness is expensive. If we are going to be fair and equitable in what we do, that is going to cost something, and we are not clear collectively about how much we are prepared to pay for that.

There are always going to be these kinds of tragic choices in health care. In dialysis originally they resulted from the fact that we could not treat all of the patients who could benefit from that treatment and, hence, we had selection committees that chose who could live and who would not. Now, the tragic choices that we are beginning to confront in regard to dialysis result not from the fact that we cannot treat everybody, but from the prospect that we can. The cost of doing so is substantial, and we are beginning to wonder whether the benefit justifies the cost, especially as we realize there are other diseases that medical progress will address, particularly as research advances an unending flow of increasingly sophisticated treatments that will work at increasingly great expense.

Now, I want to talk about transplantation, because it does seem that the best bet at the moment is increasing the proportion of end-stage renal patients who are transplantable. We have heard that one of the main problems is the problem of supply of transplantable kidneys, especially now that there does seem to be a handle on the immunological rejection problems.

There is one very simple way to solve the problem of supply of transplantable kidneys, and that is simply to enact legislation making it required that people at death have their kidneys available for medical research or medical treatment. But that solution, which is the most efficient, constitutes an intrusion in individual liberty and individual rights.

It is, again, a tragic choice, tragic in the sense that we see in conflict our desire to make transplantable organs broadly available to meet the need and, at the same time, we recognize that the most efficient way of doing that violates deeply felt commitments about individual freedom and individual rights.

We need then to explore middle ground compromise positions where the Government may play a catalytic role in increasing the availability of transplantable organs by noncoercive means. Some of the proposals that have been suggested link donor willingness with social security registration. In this country, the burden of proof is such—or the burden of responsibility is such—that you

have to take positive action to be a donor. It has been suggested that everyone should have the right to decline, but the burden should fall on one who wishes to decline; that the presumption should be with the donor. It is not compulsory. It is just a question of shifting the burden of responsibility. A lot of people are feeling very uneasy about that because it seems implicitly coercive.

The question that I urge you to consider in respect to transplantation specifically is: What appropriate role can a Federal agency, Congress, or some other Federal agency, play in striking the right sort of balance to increase the incidence of donation without being overly coercive, overly intrusive on individual rights?

There are easy answers to all of these problems, and they are nearly always wrong. One wrong, easy answer, I suggest, is making it legally required that everybody be a donor, like it or not. The problem is that the moral sensitivities that we have lead us to reject all such easy answers.

As individuals, we may hope to avoid these tragic choices if we lead very lucky lives. As public officials, that is not available to you. The burdens of public office are especially great, with government not strictly in the business of distributing benefits—which used to be more tenable than it now is—but in the business of limits and deprivations as well.

Collectively and through our governmental instruments, alas, we must develop the capacity to say "No"—not merely to look to the future hopefully and increase our support for research, but to recognize that in the final analysis, there must be limits. We simply cannot move uncritically to an era in which half the gross national product is invested in health care, half is invested in education, half is invested in defense, and so on. How we as a society ultimately will make these choices, of course, will determine and will be a reflection of the kind of society that we are.

I would hold that our concerns with equity, with compassion, with the least advantaged and, in particular, those who are medically least well off, should play a central role. But reflection about these questions is not an inquiry into practical medical problems. It is an inquiry into the very value structure of a pluralistic society.

I said at the outset that I welcomed the opportunity to be included on this panel. If I would end with one urging, it is that issues of this sort are typically included in our deliberation, because I think that when we ask narrowly how can we improve the quality of medical care for renal patients, we are asking an important question, but a somewhat parochial question, and we need more broadly to incorporate in our public forums of debate and in our educational systems serious sustained reflections about our goals societally, our national aspirations, and the ways in which we are going to solve the conflicts of value that will give us an unending supply of tragic choices to face individually and collectively.

Thank you.

Mr. GORE. Thank you very much. I appreciate that. The questions you raise are ones that we are all going to be wrestling with more and more in the future, particularly as medical science gives us more, more and more options.

We had a hearing last week on genetic screening, Congressman Shamansky and I did. And in a few weeks—well, I guess in about a

month, we have a hearing on gene therapy, the first appearance of human genetic engineering and some of the choices that you describe are very sharply posed questions in that field.

Let me ask a couple of questions to each witness, if I could.

Dr. Cerilli, first of all, you used a figure of $1,000 for maintenance of a patient after a transplant. Maybe we can clear this up fairly easily, but the figures that I have from the Health Care Financing Administration are significantly higher than that. According to HCFA, as we call it, a successful transplanted patient incurs costs of about $45 to $103 per year following transplantation for drugs and other items not reimbursed by medicare and approximately $350 a month for medical and hospital care.

Now, that is on the order of $4,200 to $4,300 per year. Where does your $1,000 figure come from?

Dr. CERILLI. I think the difference is that many of their costs were involved in patients whose grafts failed. And I included those under dialysis costs.

The $1,000 were for patients whose grafts succeeded and basically had very smooth posttransplant costs. Those really were figures conglomerative of analyses of groups of patients that we did.

And I recognize that is very difficult to define what costs are ascribed to successful transplant versus the unsuccessful.

Even taking your figures, though, which I think are high, the discrepancy is still enormous.

Mr. GORE. These are not my figures. These are the Health Care Financing Administration's and there may be some misunderstanding, but they appear to apply to successful transplant patients.

Dr. HEBERT. Could I respond?

Mr. GORE. Yes, Dr. Hebert.

Dr. HEBERT. The typical followup, for the patient, say, between 2 years after the transplant—2 years and beyond, the typical followup is the patient is seen about three or four times a year. He will then incur a physician's fee, which is usually about $25, and so that is four times $25 is $100, OK. And then we will have blood tests roughly every month, sometimes every 2 months, depending upon the level of stability. The blood tests, for example, at University Hospital, your profile, your CVC, would come to about $25 or $26. Once a year you might get a chest X-ray, get an EKG and you are talking about monthly, clearly lab costs of at most, what, 50 times at most, 50 times 12, $600, and physician's fee of $100, and easily—and medication costs are not going to be $300, so for the dominant posttransplant patient, the yearly costs are going to be less than $1,000 a year clearly, at least in central Ohio.

Where that $4,000 figure comes from, they must be adding in all which is the exception rather than the rule.

Mr. GORE. Dr. Ferguson.

Dr. FERGUSON. I do not think the problem is with comparing the two figures because you might be looking at apples and oranges. Ninety percent of the problems after transplant that require readmission or that are costly are going to happen within the first 6 months. If you look at maintenance therapy and you define it as the first 6 months, you are going to have a whole different cost than from 12 months to 18 months posttransplant. But 12 to 18 months is probably going to be not too unlike 24 to 30 months so

that, you know, the curve is one that goes down and levels off. And that level-off point, no matter what you look at, patient and graft survival, cost readmission complications breaks at about 6 months.

If HCFA is taking the first year posttransplant, they are actually going to come up with a higher figure because it is going to include readmission rates. You have got to compare same time frames.

Mr. GORE. OK. There are several disincentives to transplantation at the present time. The one clearly is the availability of kidneys to be transplanted. The second is—a second disincentive is the high rejection rate which is in the process of being changed by this major new discovery that holds down the rejection rate.

Are those by far and away the two greatest disincentives?

Dr. CERILLI. I think there are some others. As a matter of fact, I did not get a chance to—and it brings on, I think, you might say, some unfortunate human factors. If you look at relative utilization of ESRD services, in Great Britain and compare it to the United States, Great Britain having a tremendously different mechanism for funding as we both know. In the United States, about 79 percent of the patients with end-stage renal disease are treated with center dialysis across the board, and this, in fact, was a study that a cohort of Dr. Ferguson's reported at the International Transplant Society meeting a few months ago in England.

With the United Kingdom, 19 percent of their patients are treated by center dialysis. Now, there are many differences, obviously. And I am not proposing that this is the only reason, but this country functions on a fee-for-service basis, a system which I happen to believe in, to be perfectly candid with you, but nevertheless, we function on a fee-for-service basis. They function on a basis of a fixed budget for their center. They get so many dollars and they have to use those dollars in a manner which they see most appropriate.

They can keep patients in the center. They can take those patients and perform transplantation or they can put them in the home. And because at least I think in part——

Mr. GORE. Put them in the home on dialysis?

Dr. CERILLI. On dialysis.

Mr. GORE. Did they have criteria for just rejecting the patients for care?

Dr. CERILLI. Yes. Unfortunately, one of the bad spinoffs of this system is that the acceptance into their treatment programs is much more restricted than it is in this country. They will not take on the high-cost, high-risk patients, the diabetics, for instance, the elderly, for instance, like we do here. But this is such an enormous discrepancy, and I think one has to look at whether or not this might be, in part, contributing to the malflow of patients into one modality versus the other. And to mention——

Mr. GORE. Well, before you leave that chart I mean I do not want to pass over it without highlighting the significance of the fact that unlike the United States, they decide that some people are going to die because they cannot afford to keep them alive?

Dr. CERILLI. Yes, I think that is true. I think they do not accept the higher risk, higher cost type patient in their program as freely as we do. I know that is true. It is exactly right.

Mr. GORE. I am glad you prefer the other option.

Dr. CERILLI. Yes I do, but nevertheless, some amalgamation of these two systems might conceivably be the best route to go.

Now, there is another—disincentive to transplantation and that is that the new reimbursement system for dialysis that has been proposed under the new regulations. I think these new regulations will do just the opposite of what we all want, and that is to encourage transplantation.

The reimbursement system that HFCA is proposing is a composite between home and center dialysis. Basically, the centers will maybe lose a little money or break even on the center and make money on the home patients. Obviously, in order for those centers to stay functioning, particularly the smaller center, they face an enormously difficult question. It is in their financial interest to put patients into the home but those are the same general patients that do very well with transplantation, and it is not in their financial interest to steer those patients toward transplantation.

So you do not need financial incentives for transplantation. The system will take care of that. The science will take care of that.

But I think it behooves us to be careful that we do not build any financial disincentives for patients going into the most cost-effective form of therapy.

Mr. GORE. Thank you. Mr. Shamansky.

Mr. SHAMANSKY. Doctor, at this point, I keep getting back to my thoughts earlier on the actions of a tissue committee or something to avoid the subconscious pressure on a practitioner of medicine—I am a practitioner of law—to serve his own ends in good conscience. I mean, sure, this is a good system for the patients and so on and so forth. And we need a set of criteria and I pursued that with Dr. Salans and we need somebody to say, "Hey, you are violating the criteria."

How do we do that?

Dr. CERILLI. You are absolutely right. And unfortunately, what has happened is that the system to accomplish that is being weakened and stripped. Namely, the network system which is very cost effective—it is costing the Government about 2.5 percent of the whole ESRD program.

Mr. SHAMANSKY. What is the network system?

Dr. CERILLI. The network system is that the country is divided up into 30–32 networks. And a group of physicians, nurses, paramedical people, lay people get together to try to manage the ESRD program in terms of certification of new units, peer review, looking at results, looking at some problems. And the system is being stripped.

Mr. SHAMANSKY. Why? How? Who is stripping it?

Dr. CERILLI. It was being stripped by the administration, really, who says that——

Mr. SHAMANSKY. All right. I understand then that is a problem we have had continuously and I am sure we expect to have.

My question to you, then, is: What is the profession doing about it to respond to that? Where is the American Medical Association? I am just not picking them, but they are the predominant medical association. Where is organized medicine?

Dr. CERILLI. It is not there?

Mr. SHAMANSKY. Why not?

Dr. CERILLI. It should be.

Mr. SHAMANSKY. I know it should be, but what prevents it?

Dr. CERILLI. I think what prevents it is a historical philosophy for allowing independent judgment for patient care for the physician.

Mr. SHAMANSKY. How about academic medicine? Where is it? Is it not organized in its own associations?

Dr. CERILLI. Academic medicine functions with a peer review system which is almost internal. You know, they are——

Mr. GORE. He is talking about academic medicine as a political voice.

Mr. SHAMANSKY. As an independent voice.

Dr. CERILLI. The major group speaking for transplantation is the American Society of Transplant Surgeons, of which I am past president, and in that capacity I have spoken to congressional groups like yourself in the past that we are trying to do just this.

Mr. SHAMANSKY. Are you getting anywhere with the other organizations of organized medicine because you represent a part of their income?

Dr. CERILLI. No.

Mr. SHAMANSKY. But there must be—it is difficult for me to imagine that among those thousands there are not other voices independent of—believe me. I do not consider everybody venal. Otherwise, I would not be here.

Dr. CERILLI. For instance, I think it is not illogical if you have a peer review system on patient care and an individual is deemed a transplant candidate by definable criteria, that possibly some form of coverage limitation be placed on dialysis to encourage the flow of patients into transplantation. That is a type of peer review.

Mr. SHAMANSKY. Do patients not generally prefer a transplant?

Dr. CERILLI. Yes, they do, I think if they are in an environment where they see its advantages.

Unfortunately, many dialysis programs are so peripherally located from large centers that the advantages are not presented to them either verbally or by example. And that was the issue that Dr. Hebert was referring to about the dangers of the proliferation of small units scattered all over. We have little islands of sequestered patients who do not have access to the whole spectrum of patient care.

Mr. SHAMANSKY. What type of help do you get from the kidney foundation in this area of educating the kidney patients?

Dr. CERILLI. They try. They have certain brochures they pass out and they try. And there was—HCFA was putting together a pamphlet on patient education that was to be distributed. I am not quite sure of the status of that. HCFA has been demoralized for its role.

Mr. SHAMANSKY. Demoralized? You mean not to pursue a more efficient method of utilizing its funds?

Dr. CERILLI. That is right.

Mr. SHAMANSKY. Why?

Dr. CERILLI. Because the funding has been cut, their staff has been moved around, the historical organization of HCFA has been undermined so that they have been amalgamated under medicare.

Mr. SHAMANSKY. So in effect, you are saying that it is—you are cutting back on the very outfit which is designed to save money?

Dr. CERILLI. That is right.

Mr. SHAMANSKY. So it is a penny-wise and pound-foolish operation?

Dr. CERILLI. There is no question about it.

Mr. GORE. Let me ask you, Dr. Hebert, a question about your statement on spreading out the centers.

I represent a rural district that is 10,000 square miles, and there is a very real problem that dialysis patients have when they have to travel 2 hours each way several times a week to get dialysis treatment. And surely, that is a competing concern with the one you expressed, would you not agree?

Dr. HEBERT. Oh, absolutely. I am referring to a different situation where well within an hour's drive of major centers, you know, with easy flow of patients into transplantation, home dialysis, alternate therapies and so on, connected by superhighways and what have you, people would like to have dialysis centers for relatively small number of patients.

Interestingly, very often, the persons leading the bandwagon for that have been the local politicians. They see it as a status symbol for their hospital and the community and so on. They don't realize that it is in nobody's long-term interest.

The area you have described is quite different. There really is no choice. If we are going to provide these services, they will have to be small, dispersed units. The levels of care will be lower, the levels of surveillance, and so on.

Their interaction with transplant programs will also be less. But that is a necessity. These other ones are nonnecessities.

Mr. GORE. Now, in order to encourage the availability of kidneys, you mentioned, Dr. Gorovitz, a negative option. In Tennessee we have a checkoff place on everybody's drivers license, and you check either yes or no, "I will be a donor," or "I will not be a donor." And that has been a model program that has met with some success.

I don't know how you would really put into effect a negative—a true negative option unless you said that it was OK so long as the cadaver didn't object.

Dr. GOROVITZ. Yes, I think that is right, a negative checkoff system is a more fallible system, because the burden rests with the dissenter and the circumstances don't always invite dissent. I share your misgivings and I don't favor such a program.

I think that even the license checkoff system, which we have also—on my license it just says "donor" at the bottom—apparently is not sufficient to generate a supply that is adequate even for present needs, and we have been given lots of reason today to believe that the needs are going to increase very rapidly as the state of the art improves very rapidly.

That suggests that some kind of catalytic effort to shift the percentages of people who respond positively has got to be undertaken somewhere. And it seems to me that those who are concerned with the Federal cost burden of the dialysis program are, by that fact alone, appropriately concerned with efforts that would facilitate increasing the percentages of patients handled by transplantation,

whether it is through public education programs or supportive research into how to allay the fears and anxieties that prevent people from signing on as donors. But it doesn't seem to be an item that is high on the agenda of any public agency that I am aware of.

Dr. CERILLI. The fragility of the system is frightening. There was a recent series of articles, I think two, or possibly three, in Great Britain about some incident with a donor—untrue, but interpretable in the public's eye as being maybe badly handled, but it wasn't.

Organ donation dropped 60 percent and it has stayed down for several months. So it is a very fragile base upon which this whole concept is based.

Dr. FERGUSON. I think you also have to understand the system and the example you brought out in the drivers license.

In the program in Minnesota that I was involved with for 10 years, who also has and had very early on, a drivers license designation, there has never been and probably will never be a kidney harvested from a brain-dead individual from an auto accident based on his driver's license. It doesn't happen.

You always, always get consent from the next of kin in the family. That isn't the way the system works. That brings it up. That makes people think about it. It tells the brain-dead individual's family that, yes, he has thought about it enough to check it off on his driver's license, but it gives no one permission to take his organs.

Mr. GORE. I didn't realize that.

Dr. FERGUSON. That is the system and perhaps that is why there is a difference between simply putting the thing on your driver's license card and not seeing immediate results.

Mr. SHAMANSKY. Is that because of an oversight in the law, or just a hesitancy on the party——

Dr. FERGUSON. I don't think it is an oversight in the law. I think it is a very complex social/legal/ethical circumstance.

Mr. SHAMANSKY. Well, are you suggesting that we make a checkoff an explicit donation?

Dr. FERGUSON. No. I think what we suggest is the burden of checkoff is on those that don't want to donate and having public opinion assume that donation is the reasonable thing to do, and then instead of us having to go out and get permission from the next of kin for donation, the next of kin would have to come and give permission not to.

Mr. SHAMANSKY. Of course, Dr. Gorovitz just preferred the other way around?

Dr. FERGUSON. No, I don't think so. Did you?

Dr. GOROVITZ. Yes, I did. I don't advocate the negative checkoff because I think that the emotions that people have in regard to the issue of the disposition of bodily remains run very, very deep. It goes far beyond simply a question of whether a preponderance of reasons favors or opposes donation. It is not an issue that many people approach at the level of economic or rational analysis but, rather, it is something that taps into very deeply felt fears and feelings.

Our respect for individual liberty and the individual's right to exercise jurisdiction over the disposition of his remains are an important right and an important respect to maintain, so what I

would advocate is a vigorous, adequately funded, coordinated program which would enable us to understand better what motivates donors to become donors, and what deters the uncertain from becoming donors, and what can encourage a greater public awareness of the appropriateness and importance of this kind of action.

Mr. SHAMANSKY. I would like to comment. I realize one is conscious, one is not. We have in this country a blood donor program, so there is an acceptance on the part—demonstrable acceptance to giving up your blood and, say, well, that is the person consciously doing himself and taking himself in.

We are talking about a very much smaller proportion in terms of numbers.

Dr. GOROVITZ. If I may say so, there is an important difference in kind between what is from the personal point of view a renewable resource and replaceable one and what isn't.

Mr. SHAMANSKY. I am just suggesting that there is such a thing as educability of the public, however you want to make the fine distinction.

Dr. GOROVITZ. It takes educators.

Dr. CERILLI. In France, and I think possibly Italy, as well, they tried the negative approach.

Dr. GOROVITZ. France.

Dr. CERILLI. France.

What is your understanding of what happened?

Dr. GOROVITZ. I don't know.

Mr. SHAMANSKY. Can I ask a question of Dr. Hebert, if I may, just a moment?

Implicit in this whole dilemma is the fact that we are going to get back to, I am afraid, the very logic of the numbers, back to those committees again, where you thought there was a solution. In fact——

Dr. HEBERT. A solution in my presentation, I suggested the solution to the large numbers of—solution to provide dialysis and having the Federal Government support it. And I was saying that was not a solution. I am sorry. I spoke so quickly. I wanted to get my testimony in.

Mr. SHAMANSKY. You thought at that time that was a solution but, in fact, we realize it is not now a solution because of the numbers?

Dr. HEBERT. It is a monster.

Mr. SHAMANSKY. And yet, you were kind enough to take me on a tour, and I saw people of advanced age on acute dialysis. And I am not saying that they—obviously, it raises the question, we are engaging in heroic measures, aren't we?

Dr. HEBERT. I believe the patient—the one patient, maybe Dr. Bay, can comment on it. I think that patient had an acute renal failure. In other words, there was good promise that his kidney function will come back. He is being supported.

Generally with old, debilitated patients, we would take the family aside and say, "Gee, I really think his best long-term interests are"——

Mr. SHAMANSKY. In other words, just because—all right. I think you have answered my question. You would not push everybody onto that kind of thing?

Dr. HEBERT. Oh, absolutely not. We make very clear choices and, you know, we talked about pressures within an academic setting. You know, I may not be a very holy person, but I am surrounded by people who, if I behave in some nonstandard renegade fashion with respect to dialysis or whatever, I would be constrained in other ways.

So, there is a real winnowing and burnishing effect in an academic center that is not present in communities where there is, say, only one nephrologist or two nephrologists in their separate practices. Sometimes things sort of grow like topsy in those settings.

Again, I don't mean to be venal or evil. It is a real problem. There are lots of patients who go on to dialysis who have donors, have family donors, and those patients eventually get a transplant from a family member. But my goodness, 6 months, 9 months, a year and a half, and one thing switches into another. Of course, the longer you stretch these workups out before the patient finally gets the transplant, the larger is the pool of patients on dialysis.

I think we need some national performance standards which say that when a patient with known living donors goes on dialysis, if that patient does not have a transplant within 6 weeks or 3 months or what have you, that there should be some explanation made; and if this happens too often, that you talk of accreditation and what have you.

Dr. CERILLI. You know, in Great Britain, this very question, they address it by being very tough. When they ran over budget, they cut their budget. They literally closed units that overspent their budget. They very definitely had limited patient numbers. There is no question about it. That is how they handled it.

Their other thing is, as you know, their educational system limits the number of physicians at a specialty level, which tends to, I think, cluster patients in centers because you are limiting the number of physicians providing that service. They cluster in centers and you minimize some of the risks and problems that Dr. Hebert mentioned.

Dr. FERGUSON. Could I say one thing about going back to the donors and picking up on a point that Dr. Hebert made that I think is terribly germane and, perhaps, not totally understood.

A donor is not an individual that arrives DOA in an emergency room. Cannot be used. The kidneys are dead, just like the rest of his body.

Mr. SHAMANSKY. I didn't realize that.

Dr. FERGUSON. That is the point I was trying to make. When I said to give the example I brought up, a brain-dead individual, brain death means that his brain is dead and that his heart is beating, he has a blood pressure, he is being supported, he is making urine, his kidneys are working, it is just his brain is dead.

If public acceptance of brain death and that patient and the lack, perhaps—a controversial issue—perhaps the lack of a legal, uniform Federal definition of brain death that then brings up a point about: Is it a legal question that is limiting donations?

If there was, in fact, a Federal law that defined brain death as such, then the patient would be dead.

Mr. SHAMANSKY. Of course, traditionally that wouldn't be a Federal issue. That would be a State issue.

Dr. FERGUSON. But each State is doing it differently. I think the other thing that Dr. Hebert—well, it is not important. It is just fine.

Mr. GORE. Let me say that to a limited degree it is an issue of a Federal dimension. The President's Commission on Biomedical Research, Bioethics—I forget the official title of it—you probably know it——

Dr. GOROVITZ. It is another grotesque title. It is the President's Commission for the Study of Ethical Problems in Medicine and Biomedical and Behavioral Research.

Mr. GORE. Buckeye, we call it.

They recently had a report on brain death, and a definition recommending that it be used in the jurisdictions where Federal law prevailed, such as the District of Columbia, the territories and so forth, and we have been considering a hearing on that particular minefield for some time later on.

Let me ask any of you whether or not there has been significant research on the development of an artificial kidney. Anybody know of such research?

Dr. HEBERT. There is no meaningful effort. People have sort of conceived of one, but when you think about what the basic problem is, to take things out of the body, it really isn't practicable to implant something to do all those filtration processes.

Mr. GORE. You don't even know exactly what it takes out and what it puts in yet?

Dr. HEBERT. Well, we don't know exactly what it is, but we know what it runs with—the urea in the blood. So if you get the urea out, you generally get out all the things that cause uremia. Probably there is not one human toxin but a whole host of things relating to retention of waste properties.

Mr. GORE. Is it possible that an artificial kidney would be feasible at some point in the future?

Dr. HEBERT. I don't think so.

Mr. GORE. It is just too complex an organ?

Dr. HEBERT. There is no way—yes, an implant. You know, we sort of kid in nephrology that the heart is sort of a dumb organ. All it does is go lubb-dubb and pumps blood, and we think of it simply as being a kidney pump.

Whereas the kidney is sort of a thinking organ. It filters, reabsorbs, makes hormones, it excretes acids and so on. It is a very complex organ, very much unlike the heart or the lung, which are fairly simple conceptually.

So, I really don't think that that is feasible. That would not be a future fruitful direction. I think the fact that there is no research in that direction sort of speaks to the issue.

Mr. GORE. I once read a book on, what was it, Fish to Philosopher?

Dr. HEBERT. Homer Smith was the physiologist.

Mr. GORE. Illustrating the central role played by the kidney.

Well, let me thank you all for extraordinarily good testimony. I think that the record of this hearing will be extremely helpful to the Congress as we confront the sharper and sharper questions

posed in this area. We intend to take this record and bring it to the attention of those among our colleagues who work with us on this program.

It is clear, in a preliminary way, that the importance of research in this area has been underestimated and the nature of the conflicts with which we are going to soon be confronted ought to lead us to do more and better research at institutions like this one.

In addition, it seems that Dr. Gorovitz' suggestion that government play a more active catalytic role in increasing the number of kidney donors obviously makes a lot of sense.

Dr. Cerilli, your points are very well made and taken about the possible disincentives that these new changes by the administration may build into transplantation, and we need to immediately bring this testimony to the attention of all our colleagues on the Ways and Means Committee.

Again, let me thank all of you and the other witnesses who have appeared today for what I think has been a really unusually helpful hearing.

Did you want to say something?

Mr. SHAMANSKY. Yes. I just want to say that I think we have had absolutely vivid proof of what a great institution this place is. I told you about that.

Mr. GORE. Once again.

Mr. SHAMANSKY. Thank you very much for your cooperation.

Mr. GORE. The hearing is adjourned.

[Whereupon, at 2:10 p.m. the subcommittee adjourned, subject to call of the Chair.]

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